October 6, 2020

Virtual Investor Day

Welcome

Chris Stevo,
Head of Investor Relations

Agenda

Introduction: Vision and Strategy	**Ludwig Hantson**, Ph.D., CEO **Aradhana Sarin**, M.D., CFO
Board of Directors Perspective	**David Brennan**, Chairman of the Board
R&D Portfolio Overview	**John Orloff**, M.D., Head of R&D
Lead & Expand: Sustainability in Our C5 Business	**Brian Goff**, Chief Commercial & Global Operations Officer **John Orloff**, M.D., Head of R&D
Q&A Session I	**30 minutes**
Highlighted Diversification Opportunities and Novel Platforms in our Portfolio	
ALXN1840 in Wilson Disease	**John Orloff**, M.D., Head of R&D & **Brian Goff**, Chief Commercial & Global Operations Officer
CAEL-101 in AL Amyloidosis	**Cristina Quarta**, M.D., Ph.D., CAEL-101 Clinical Development Lead
Factor D Platform (ALXN2040 & ALXN2050)	**Gianluca Pirozzi**, M.D., Ph.D., Head of Clinical Development and Translational Sciences **Anita Hill**, M.D., Ph.D., Hematology Global Medical Affairs Lead **Darius Moshfeghi**, M.D., Clinical Consultant GA Program & Professor, Stanford University
FcRn Platform (ALXN1830)	**Gianluca Pirozzi**, M.D., Ph.D., Head of Clinical Development and Translational Sciences
Internal Research & Discovery	**Sharon Barr**, Ph.D., Head of Research, Bioinformatics, & Diagnostics
Q&A Session II	**30 minutes**
Conclusion	**Ludwig Hantson**, Ph.D., CEO

Investor Day Speakers

Alexion Board of Directors & Management Team

David Brennan
Chairman of the Board

Ludwig Hantson, Ph.D.
Chief Executive Officer

Aradhana Sarin, M.D.
Chief Financial Officer

John Orloff, M.D.
Head of R&D

Brian Goff
Chief Commercial & Global Operations Officer

Alexion R&D Leadership & Clinical Experts

Gianluca Pirozzi, M.D., Ph.D.
Head of Clinical Development & Translational Sciences

Cristina Quarta, M.D., Ph.D.
CAEL-101 Clinical Development Lead

Darius Moshfeghi, M.D.
Consultant, ALXN2040 GA & Professor of Ophthalmology, Stanford University

Anita Hill, M.D., Ph.D.
Hematology Global Medical Affairs Lead

Sharon Barr, Ph.D.
Head of Research, Bioinformatics, & Diagnostics

Forward Looking Statements

This presentation contains forward-looking statements, including statements related to: anticipated financial results (including short-term guidance and long-range financial guidance), including expected increases to the revenue guidance for 2020, revenue and non-GAAP operating margin by 2025, our cumulative average growth rate through 2025, and peak revenue from our current pipeline beyond 2025 (and all of the assumptions, judgments and estimates related to such anticipated future results); expectation of multiple pivotal clinical results over the next 12 months; ambition to quadruple the number of neurology patients in the US by 2025; ambition for 10 product launches by 2023; anticipated future product launches (and the timing of those launches); the Company's capital allocation strategy and plans concerning the repurchase of Alexion shares; the anticipated amount and timing of future share repurchases by the Company; plans to make regulatory filings for approval of certain products and product candidates, the expected timing of such filings as well as the expected timing of the receipt of certain regulatory approvals to market a product; the ability of our pipeline and existing products to provide long-term sustainable growth for shareholders; Company's plans for future clinical trials and studies, the timing for the commencement and conclusion of future clinical trials and the expected timing of the receipt of results of clinical trials and studies; the anticipated number of patients that may be treated with the Company's products; the Company's strategy for long-term value creation (including the following: establishing ULTOMIRIS as the new standard of care in PNH, aHUS and Neurology, plans to launch our next generation C5 formulations, plans to expand our presence in Neurology, focus ULTOMIRIS expansion on direct to phase 3 rapid proof of concept, grow acute care presence with ANDEXXA and execute novel asset development to expand our rare disease focus); plans to further diversify our assets and establish novel platforms and the benefits of those plans; plans to establish 7 blockbuster franchises and the targeted indications in each franchise; potential peak sales of our pipeline assets; the upcoming catalysts for our pipeline; plans to innovate and remain at the forefront of C5 science; plans for additional formulations of ULTOMIRIS (high concentration and subcutaneous) and the timing for regulatory approval and potential benefits of such formulations; potential launches of ULTOMIRIS for additional indications and in additional countries, including for ALS; plans and anticipated timing to launch ALXN1840 in Wilson disease; the development of a new biomarker for Wilson disease to detect labile bound copper and the potential benefits of such biomarker; the ability of ALXN1840 to redefine treatment goals; plans for the development and launch of CAEL-101 as a treatment for AL-Amyloidosis and the ability of CAEL-101 to transform the treatment landscape for AL-Amyloidosis; the development plans and potential indications for ALXN1820 and ALXN1850; the anticipated clinical trials for our late stage pipeline including the start and end times for such trials; the anticipated events that support the three pillars of Alexion's value creation strategy, including regulatory approval for ULTOMIRIS high concentration and subcutaneous formulations and the initiation of certain clinical trials; Alexion's ambitions for its portfolio of assets; the anticipated pricing of ULTOMIRIS in PNH and aHUS; the affected patient populations in the indications we are pursuing; plans to develop and launch ALXN1720 in gMG and DM; development and commercialization plans for ALXN2040 and ALXN2050, including in PNH, geographic atrophy and renal diseases, and the potential benefits of those therapies; development plans and the value proposition for ALXN1830; plans for our CSR program for the 2nd half of 2020; the growth potential of our FcRn program; continued diversification of the pipeline; and the estimated addressable patient populations for our current and pipeline products. Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ materially from those forward-looking statements, including for example: our dependence on sales from our C5 products (SOLIRIS and ULTOMIRIS); delays (expected or unexpected) in the time it takes regulatory agencies to review and make determinations on applications for the marketing approval of our products; Alexion's inability to timely submit (or failure to submit) future applications for regulatory approval for our products and product candidates; payer, physician and patient acceptance of ULTOMIRIS as an alternative to SOLIRIS; appropriate pricing for ULTOMIRIS; future competition from biosimilars and novel products; inability to timely initiate (or failure to initiate) and complete future clinical trials due to safety issues, IRB decisions, CMC-related issues, expense or unfavorable results from earlier trials (among other reasons); the number of patients that will use our products and product candidates in the future; decisions of regulatory authorities regarding the adequacy of our research, marketing approval or material limitations on the marketing of our products; delays or failure of product candidates to obtain regulatory approval; delays or the inability to launch product candidates due to regulatory restrictions, anticipated expense or other matters; interruptions or failures in the manufacture and supply of our products and our product candidates; failure to satisfactorily address matters raised by the FDA and other regulatory agencies; results in early stage clinical trials may not be indicative of full results or results from later stage or larger clinical trials (or broader patient populations) and do not ensure regulatory approval; the possibility that results of clinical trials are not predictive of safety and efficacy and potency of our products (or we fail to adequately operate or manage our clinical trials) which could cause us to halt trials, delay or prevent us from making regulatory approval filings or result in denial of regulatory approval of our product candidates; unexpected delays in clinical trials; unexpected concerns that may arise from additional data or analysis obtained during clinical trials; future product improvements may not be realized due to expense or feasibility or other factors; uncertainty of long-term success in developing, licensing or acquiring other product candidates or additional indications for existing products; inability to complete acquisitions due to failure of regulatory approval or material changes in target or otherwise; inability to complete acquisitions and investments due to increased competition for technology; the possibility that current rates of adoption of our products are not sustained (or anticipated adoption rates are not realized); internal development efforts do not result in commercialization of additional products; the adequacy of our pharmacovigilance and drug safety reporting processes; failure to protect and enforce our data, intellectual property and proprietary rights and the risks and uncertainties relating to intellectual property claims, lawsuits and challenges against us (including intellectual property lawsuits relating to products brought by third parties against Alexion); the risk that third party payors (including governmental agencies) will not reimburse or continue to reimburse for the use of our products at acceptable rates or at all; failure to realize the benefits and potential of investments, collaborations, licenses and acquisitions; failure by regulatory authorities to approve transactions; the possibility that expected tax benefits will not be realized or that tax liabilities exceed current expectations; assessment of impact of recent accounting pronouncements; potential declines in sovereign credit ratings or sovereign defaults in countries where we sell our products; delay of collection or reduction in reimbursement due to adverse economic conditions or changes in government and private insurer regulations and approaches to reimbursement; uncertainties surrounding legal proceedings, company investigations and government investigations; the risk that estimates regarding the number of patients with PNH, aHUS, gMG, NMOSD, HPP and LAL-D and other future indications we are pursuing are inaccurate; the risks of changing foreign exchange rates; risks relating to the potential effects of the Company's restructuring; risks related to the acquisition of companies and co-development and collaboration efforts; and a variety of other risks set forth from time to time in Alexion's filings with the SEC, including but not limited to the risks discussed in Alexion's Quarterly Report on Form 10-Q for the period ended June 30, 2020 and in our other filings with the SEC. Alexion disclaims any obligation to update any of these forward-looking statements to reflect events or circumstances after the date hereof, except when a duty arises under law.

In addition to financial information prepared in accordance with GAAP, this press release also contains non-GAAP financial measures that Alexion believes, when considered together with the GAAP information, provide investors and management with supplemental information relating to performance, trends and prospects that promote a more complete understanding of our operating results and financial position during different periods. Alexion also uses these non-GAAP financial measures to establish budgets, set operational goals and to evaluate the performance of the business. The non-GAAP results, determined in accordance with our internal policies, exclude the impact of the following GAAP items (see reconciliation tables below for additional information): share-based compensation expense, fair value adjustment of inventory acquired, amortization of purchased intangible assets, changes in fair value of contingent consideration, restructuring and related expenses, upfront payments related to licenses and other strategic agreements, acquired in-process research and development, impairment of purchased intangible assets, gains and losses related to strategic equity investments, litigation charges, gain or loss on sale of a business or asset, gain or loss related to purchase options, contingent milestone payments associated with acquisitions of legal entities accounted for as asset acquisitions, acquisition related costs and certain adjustments to income tax expense. These non-GAAP financial measures are not intended to be considered in isolation or as a substitute for, or superior to, the financial measures prepared and presented in accordance with GAAP, and should be reviewed in conjunction with the relevant GAAP financial measures. Please refer to the attached Reconciliations of GAAP to non-GAAP Financial Results and GAAP to non-GAAP 2020 Financial Guidance for explanations of the amounts adjusted to arrive at non-GAAP net income and non-GAAP earnings per share amounts for the three and six month periods ended June 30, 2020 and 2019 and for the twelve months ending December 31, 2017 through 2019 and projected twelve months ending December 31, 2020.

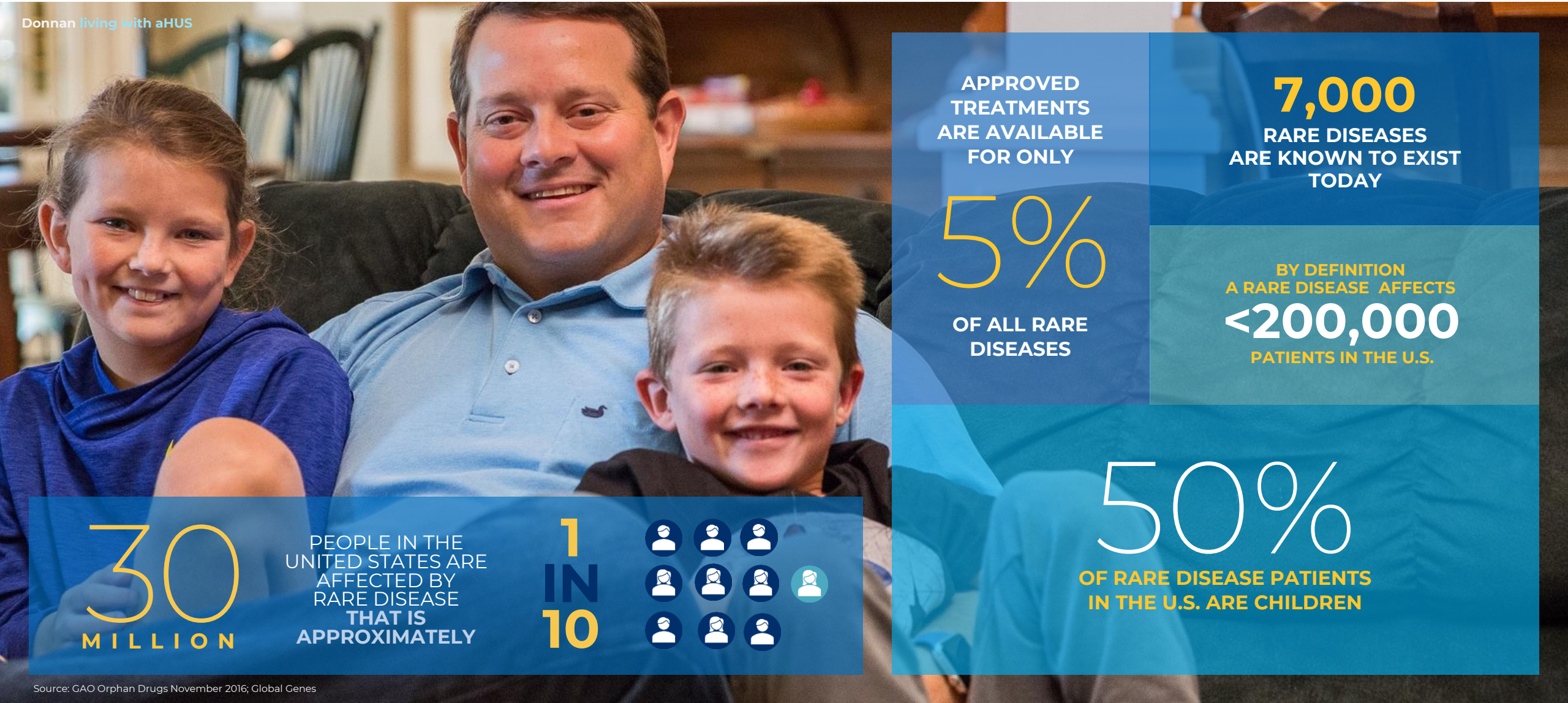
Amounts may not foot due to rounding.

Vision & Strategy

Ludwig Hantson, Ph.D.
Chief Executive Officer

Aradhana Sarin, M.D.
Chief Financial Officer

Rare Disease By The Numbers



APPROVED TREATMENTS ARE AVAILABLE FOR ONLY

5%

OF ALL RARE DISEASES

7,000

RARE DISEASES ARE KNOWN TO EXIST TODAY

BY DEFINITION A RARE DISEASE AFFECTS

<200,000

PATIENTS IN THE U.S.

30 MILLION

PEOPLE IN THE UNITED STATES ARE AFFECTED BY RARE DISEASE **THAT IS APPROXIMATELY**

1 IN 10

50%

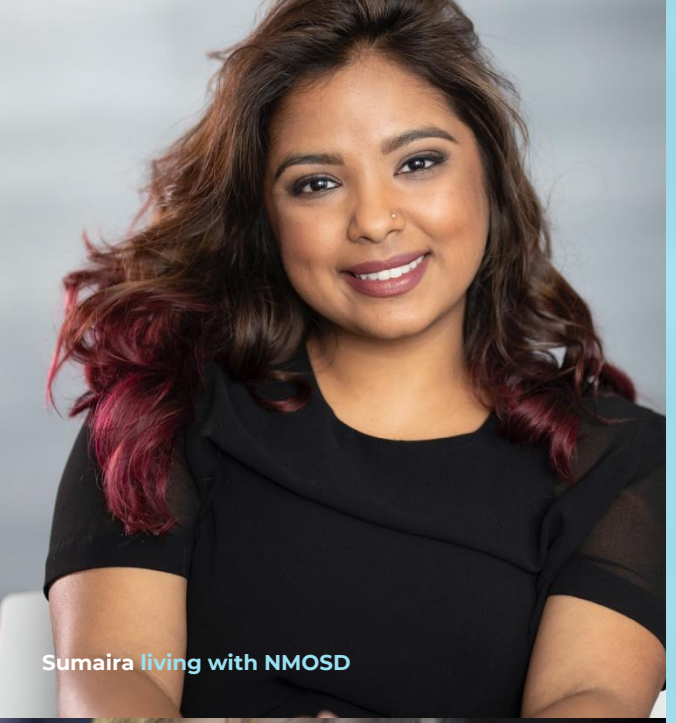
OF RARE DISEASE PATIENTS IN THE U.S. ARE CHILDREN

Source: GAO Orphan Drugs November 2016; Global Genes

Patients are Our Inspiration

Chelsey advocated for herself and was diagnosed with NMOSD in 2019

Chelsey living with NMOSD


Sumaira living with NMOSD


Albie living with LAL-D

Our Mission:

Transform the lives of people affected by rare diseases and devastating conditions by continuously innovating and creating meaningful value in all we do

Jesse living with gMG


Justice living with aHUS


Bunny living with PNH


Aira living with HPP

Committed to Our Focused Rare Disease Model

ALEXION
Focused Rare Disease Model

R&D
Internal complement innovation; committed to maximizing value of platform technologies with reinvigorated focus on speed to proof-of-concept

Commercial
High-touch patient support, innovative diagnostics, patient-centered access models, and experienced rare disease field teams

Financial Execution & Culture
Best-in-class financial discipline with culture focused on quality and compliance

NIMBLE SIZE, RARE DISEASE FOCUS AND SUFFICIENT RESOURCING

Key Takeaways

- Sustainable growth in portfolio **targeting $9-10B in revenue in 2025**[1] (current consensus estimate ~$7.5B[2]) while maintaining >50% non-GAAP operating margins
- **Current pipeline contributes >$10B in peak sales potential beyond 2025**
- Differentiated as a **biotech focused in rare disease** with resourcing capacity to reinvest in our pipeline and commercial capabilities; ambition for >5 INDs by 2025
- Robust pipeline with **terminal complement, Factor D and anti-FcRn Platforms** and **novel rare disease assets** contributing to 7 blockbuster franchises; multiple pivotal results in next 12+ months

[1] 2025 $9-10B target is at constant currencies (9/30/20 levels); [2] Consensus estimate as of Alexion Investor Day on October 6, 2020

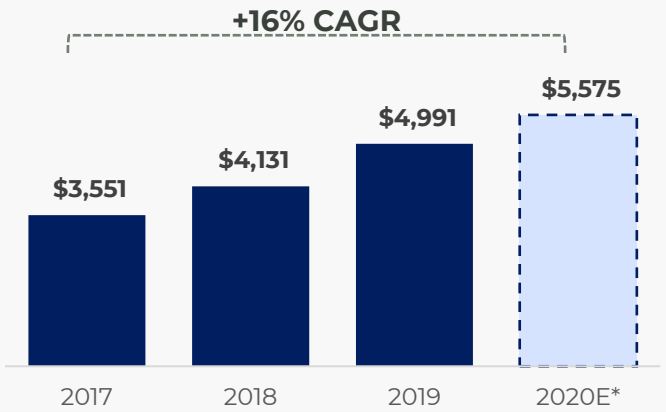
Proven Operational Excellence

Global Organization Continuing to Innovate for Patients

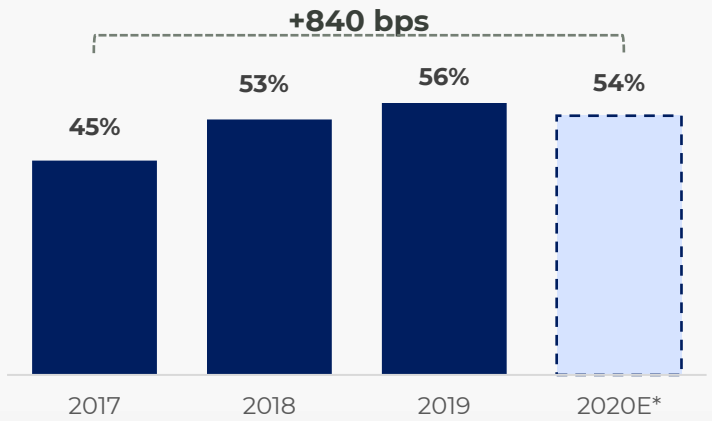
- Established ULTOMIRIS as **PNH Standard Of Care** Across Major Geographies (US, DE, JP)
- Executed **12 Business Development Transactions**
- SOLIRIS **First Approved Therapy For NMOSD**
- Expanded Portfolio To **>20 Clinical Stage Programs**
- Demonstrated **Commercial Excellence:** Neurology Largest US Franchise After Just 2 Years
- **Transformed Patient-Centric Culture;** Focus On Compliance and Integrity

Consistent Financial Execution[1]

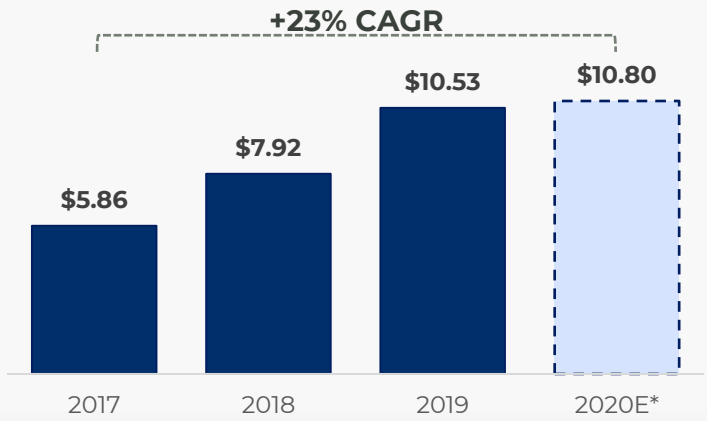
Double Digit Revenue Growth ($M)



Non-GAAP Margin Expansion +840bps

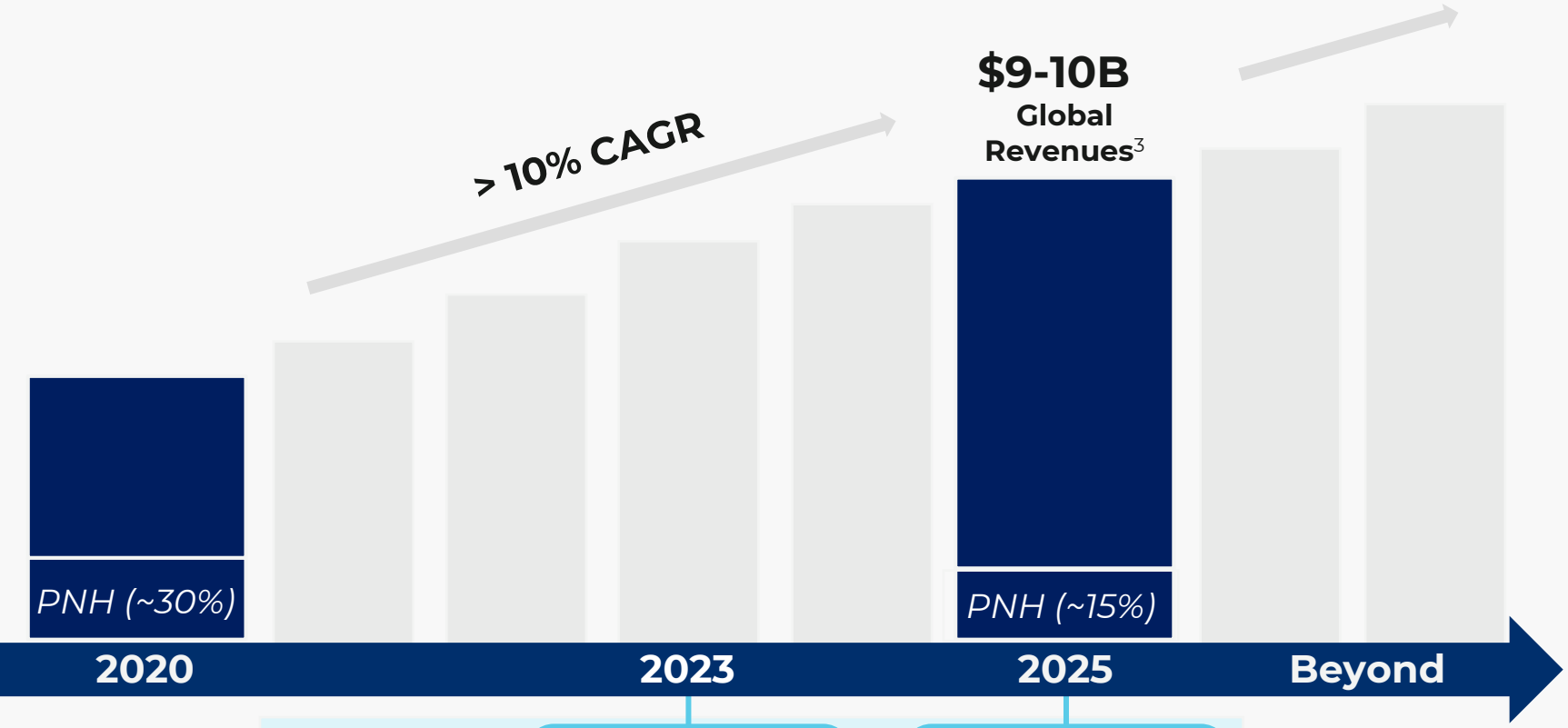


Nearly Doubled Non-GAAP EPS



[1]A reconciliation of GAAP to non-GAAP financial results is provided in the appendix and is available at www.alexion.com; *2020E based on midpoint of 2020 Guidance issued July 30, 2020. The financial guidance (and related assumptions) set forth herein was provided as of July 30, 2020 (such guidance has not been updated to reflect any events subsequent to July 30, 2020).

Targeting $9-10B in Global Revenues in 2025



Key Growth Drivers from 2020 to 2025 to Achieve $9-10B in Global Revenues

- Organic SOLIRIS/ULTOMIRIS C5 Neurology portfolio growth
- Metabolic (STRENSIQ, KANUMA) volume growth
- ANDEXXA utilization expansion

Operational Execution and Capital Allocation

- Initial revenue contribution from 10 launches by 2023
- Maintain >50% non-GAAP operating margins
- Dedicate at least 1/3 of FCF toward share repurchases

Beyond 2025

- Robust pipeline maximizing existing assets with >$10B+ in peak sales potential
- Continued financial execution and strong FCF generation allows for reinvestment

SHARE BUYBACK PROGRAM (~10% REDUCTION IN O/S) WITHOUT INCREASING LEVERAGE (~1.0X) [2]

[1]Ambition Baseline - 12/31/19 1,885 patients [2] Relative to 12/31/19 share count, excluding impact of new issuances [3]2025 $9-10B target is at constant currencies (9/30/20 levels)

Our Value Creation Strategy

LEAD AND *EXPAND* IN COMPLEMENT

DIVERSIFY INTO NEW GROWTH AREAS

LEAD

- Establish ULTOMIRIS as the new standard of care
  - PNH
  - aHUS
  - Neurology in 2022/2023
- Develop and launch next-generation innovative C5 formulations

EXPAND

- Expand presence in Neurology
- Focus new ULTOMIRIS expansion opportunities on direct-to-Phase 3, rapid Proof of Concept

DIVERSIFY

- Execute novel asset development to expand rare disease focus
- Grow acute care presence with Andexxa launch

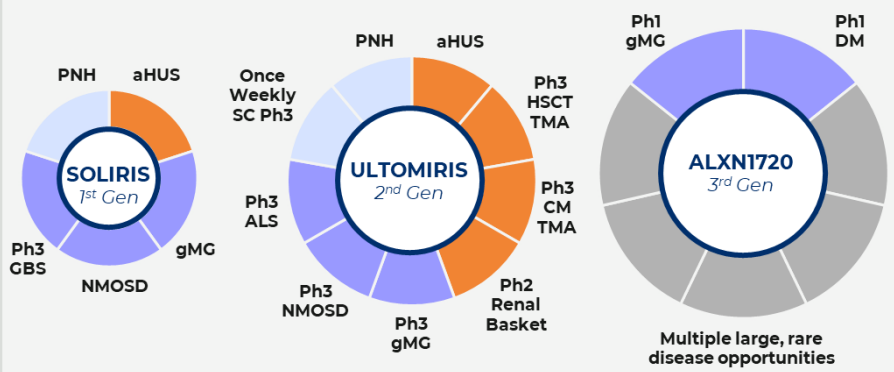
Secure and grow our base business → ***Drive new growth opportunities outside C5***

Novel Platforms & Assets Further Diversification

Novel Platforms

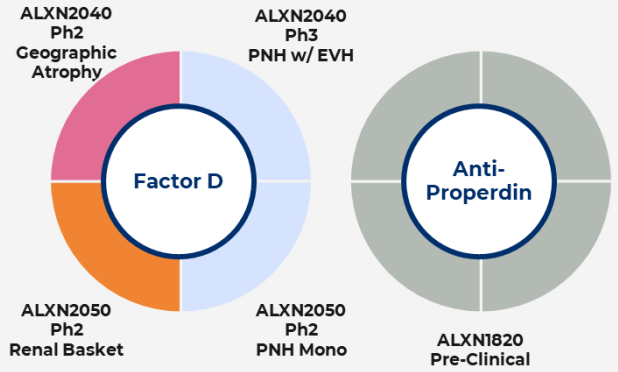
C5

Continuous Innovation in C5 through Transformative Therapies



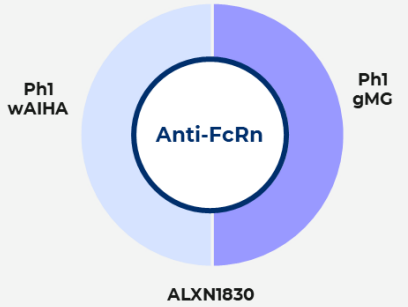
Non C5

While Widening the Complement Technology Platform



Non Complement

And Expanding Beyond Complement



Novel Assets

ALXN1840	CAEL-101	ANDEXXA	AG10
Ph3	Ph3	Ph2	Ph3
Wilson Disease	AL-Amyloidosis	Urgent Surgery	ATTR-CM

Hematology · Nephrology · Metabolics · Neurology · Cardiology · Ophthalmology · Acute Care

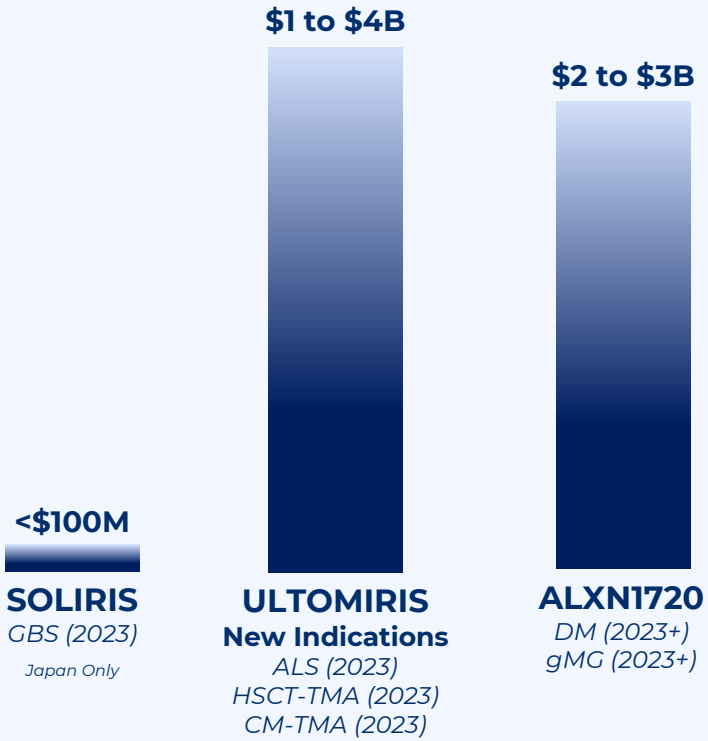
Building 7 Blockbuster Franchises

	Hematology	Nephrology	Metabolics	Neurology	Cardiology	Ophthalmology	Acute Care
Indications Diagnosed Prevalence	**Paroxysmal Nocturnal Hemoglobinuria** (PNH) Ultra-Rare[1] **Warm Autoimmune Hemolytic Anemia** (WAIHA) ~65K U.S. and EU	**Atypical Hemolytic Uremic Syndrome** (aHUS) Ultra-Rare[1] **Hematopoietic Stem Cell Transplantation**[2] (HSCT-TMA) ~5K U.S. Only **Complement Mediated TMA** (CM-TMA) ~2K Addt US Oppty **Renal Basket** (LN, IgAN, PMN, C3G) >200K U.S. Only	**Hypophosphatasia** (HPP) Ultra-Rare[1] **Lysosomal Acid Lipase Deficiency** (LAL-D) Ultra-Rare[1] **Wilson Disease** ~10K U.S. and EU	**Generalized Myasthenia Gravis** (gMG) 60-80K U.S. Only **Neuromyelitis Optica Spectrum Disorder** (NMOSD) ~10K U.S. Only **Amyotrophic Lateral Sclerosis** (ALS) >40K U.S., EU, & Japan **Guillain-Barre Syndrome**[2,3] (GBS) <2K Japan Only **Dermatomyositis** (DM) ~50K U.S. Only	**AL Amyloidosis** ~20K U.S. & EU Only **Transthyretin Amyloid Cardiomyopathy**[4] (ATTR-CM) <6K Japan Only	**Geographic Atrophy** (GA) ~2M U.S. and EU	**Factor Xa Major Bleeds** ~700K U.S. and EU **Factor Xa Reversal for Urgent Surgery** ~100K U.S. and EU

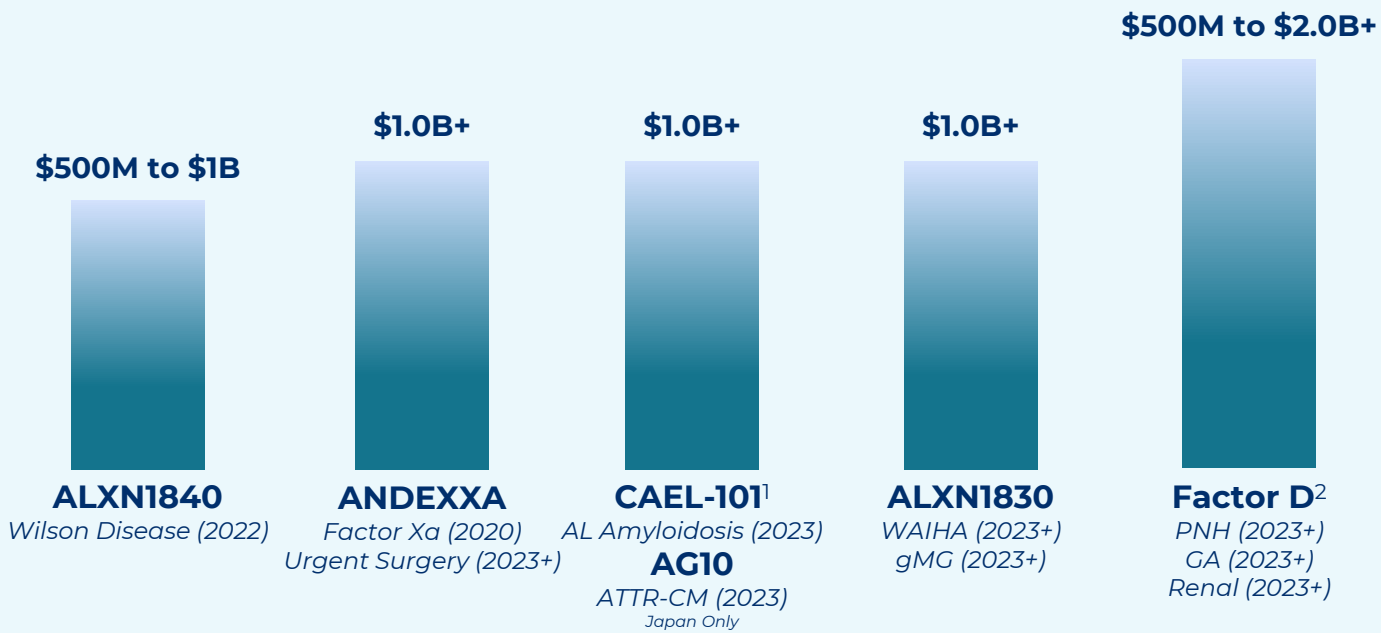
Source: Internal epidemiological analysis; [1]Ultra-Rare: <6K; [2]Incident population; [3]Currently pursuing Japanese development only; [4]ALXN has rights to Japanese development only

Development-Stage Pipeline with >$10B+ in Potential Peak Sales

LEAD and *EXPAND* in complement



DIVERSIFY into new growth areas *(sourced through BD)*



7 Blockbuster Franchises

Illustrative only; timing shown represents launch year; based on non-adjusted peak revenue estimates for incremental market opportunity; [1]Structured as an option to acquire Caelum; [2]Factor D represents both ALXN2040 and ALXN2050

Board of Director Perspectives

David Brennan,
Chairman of the Board

VALUE

GROWTH

R&D Portfolio Overview

John Orloff, M.D.
Head of R&D

Advancing a Differentiated Rare Disease Pipeline

Leading R&D Engine Supports Rare Disease Innovation

- Global regulatory team with deep rare disease expertise
- High-quality, fit-for-purpose PK/PD modeling and simulation
- Robust clinical operations capabilities leveraging Artificial Intelligence (AI) in clinical trial development
- Diverse clinical development expertise across therapeutic areas to fully leverage potential of platform technologies
- Deep complement biology supporting out internal innovation engine to unlock potential throughout complement cascade
- Patient-centered model informs clinical trial design

ROBUST PIPELINE DRIVING NUMEROUS UPCOMING CATALYSTS

2021

- ALXN1840 Wilson Ph3 TLR* (1H)
- ULTOMIRIS COVID-19 Ph3 TLR (1H)
- ALXN2050 PNH Ph2 TLR (2H)
- ULTOMIRIS gMG Ph3 TLR (2H)

2022

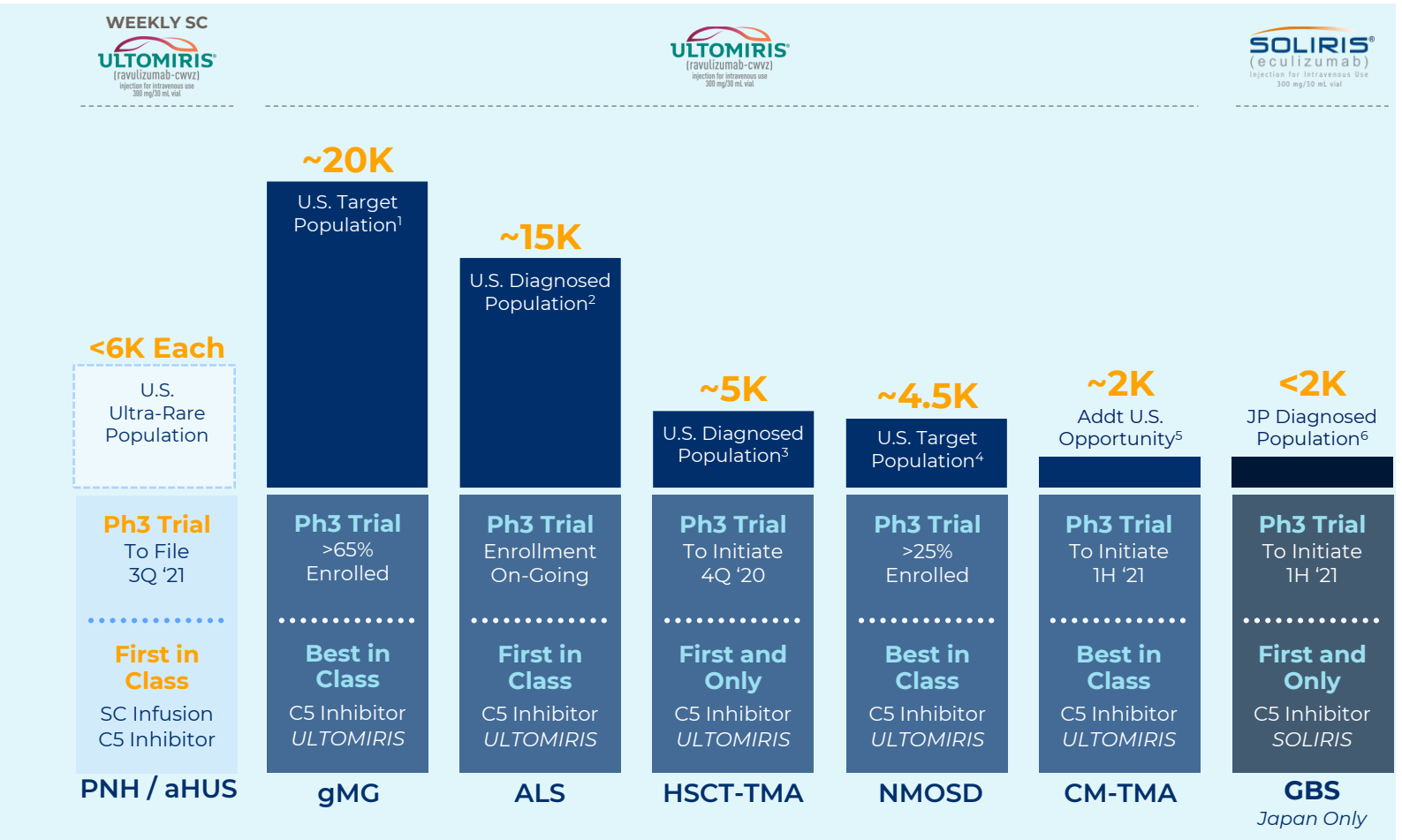
- ULTOMIRIS NMOSD TLR (1H)
- ULTOMIRIS SC PNH/aHUS launch (mid-22)
- AG10 Japan Ph3 TLR (2H)
- ULTOMIRIS ALS Ph3 TLR (2H)
- CAEL-101 Ph3 TLR (2H)
- ALXN1840 Wilson US launch (2H)
- ULTOMIRIS gMG US launch (2H)

UNIQUE CAPABILITIES DRIVE ROBUST PIPELINE WITH MULTIPLE POTENTIAL BLOCKBUSTERS

*TLR: Top line readout

Targeting 10 Launches by 2023

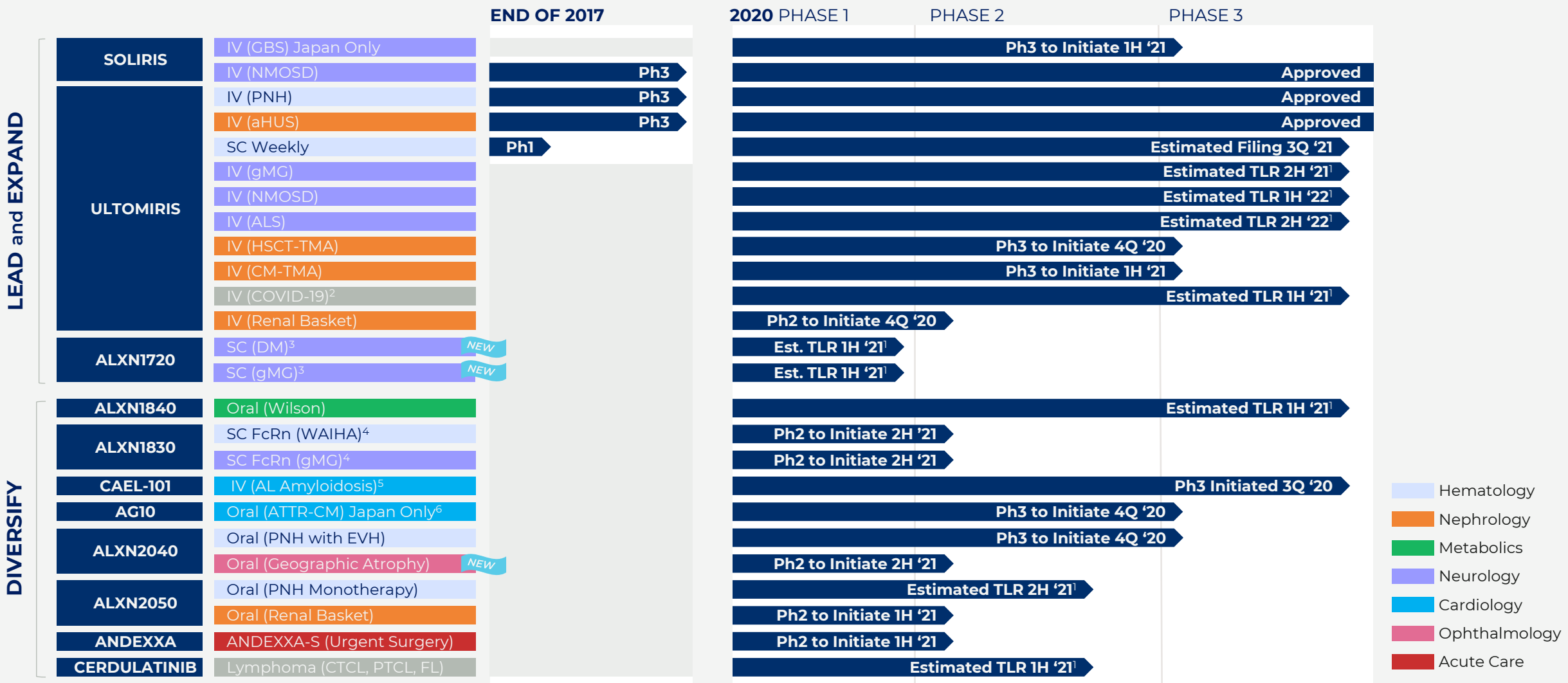
LEAD AND EXPAND IN COMPLEMENT



DIVERSIFY INTO NEW GROWTH AREAS

1. Commercial estimate 2. Prevalence of ALS-United States, 2015 MMWR Morb Mortal Wkly Rep. 2018 Nov 23; 67(46): 1285-1289 3. Jodele S, Davies SM, Lane A, et al. Diagnostic and risk criteria for HSCT-associated thrombotic microangiopathy: a study in children and young adults. Blood. 2014;124(4):645–653. 4. Aligned with our Phase 3 PREVENT criteria 5.Alexion estimated market opportunity incremental to existing aHUS market 6. Saito T, Arimura K, No M. Result report of the National Epidemiology Survey secondary questionnaire survey on Guillain-Barré syndrome, Ministry of Health, Labour and Welfare specific disease, Immunologic neurological disease investigation sub-group Year 2000 Research Report, 2000;83-84. 7. Quock, T. P., et al. Epidemiology of AL amyloidosis: a real-world study using US claims data. *Blood Adv.* 2018; 2(10):1046-1053 8. Eidos Therapeutics 9. Poujois, A., et al. Characteristics and prevalence of Wilson's disease: A 2013 observational population-based study in France. Clin Res Hepatol Gastroenterol. 2018 Feb;42(1):57-6 10. Risitano AM, et al. *Blood.*2009;113(17):4094-4100

Robust Clinical Stage Pipeline Progress since 2017



[1]TLR: Topline readout; [2]Adults with COVID-19 who are hospitalized with severe pneumonia or acute respiratory distress syndrome (ARDS); [3]1720 currently in HV Ph1 with topline readout estimated 1H '21 and subsequent DM and gMG trials to begin after that; [4]1830 Ph1 HV program to reinitiate for SC formulation with WAIHA and gMG Ph2 programs to follow in 2021; [5]Structured as option to acquire Caelum; [6]Exclusive license to develop & commercialize in Japan

Lead & Expand: Sustainability in our C5 Business

Brian Goff
Chief Commercial & Global Operations Officer

John Orloff, M.D.
Head of R&D

Commercial Model Tailored to Rare Disease

Rare Disease Focused Field Force

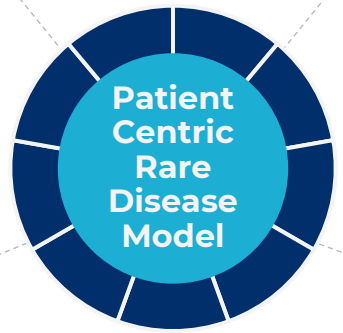
Increasing depth of experience building physician education and KOL relationships

- Specialized therapeutic area expertise
- Extensive tenure in pharma / biotech

Best In Class Data Analytics

Innovating to find alternative ways to help a rare disease patient receive their diagnosis earlier

- Artificial intelligence using de-identified claims to ID potential HCPs who may have rare disease patients



Operational Excellence

High quality, secure, and consistent manufacturing and supply

- Comprehensive global footprint
- Robust distribution & supply chain

Patient-centered Access Models

Building sustainable pricing solutions to support patient access to treatments

- ULTOMIRIS pricing strategy
- HEOR, Access, Contracting Field Support

ONESOURCE®
Personalized Patient Support from Alexion

- Individual Case Managers
- Vaccination Support
- Funding Assistance
- Home Infusion Assistance

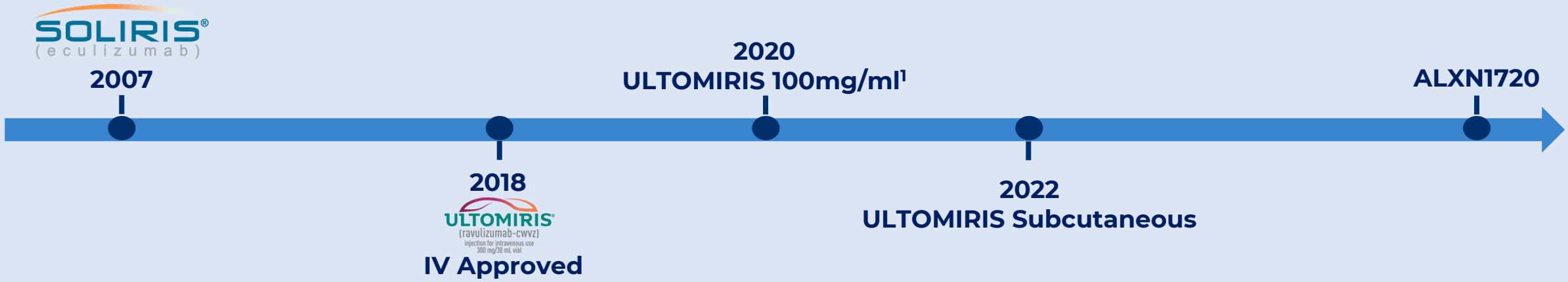
Simple and continuously improving experience for patients/caregivers to support patients initiating and maintaining access to treatment

Personalized High-Touch Patient Support Services*

RARE DISEASE TAILORED CAPABILITIES, FOCUS & GLOBAL SCALE

*OneSource is a U.S. Patient Service Model

Innovating to Remain at the Forefront of C5 Science



First generation C5
Ultra-Rare Focus
<6K Patients

Second generation C5
Expanding to Rare
>50K Potential Addressable Patients

Third generation C5
Continued Expansion in Rare
>100K Potential Addressable Patients

ULTOMIRIS Q8W IV

- Annual treatment cost per patient significantly lower than SOLIRIS[2]
  - 10% in PNH
  - 33% in aHUS, gMG, NMOSD

ULTOMIRIS 100mg/mL High Concentration

- Delivers on patient preference for shorter infusion time (45 minutes)
- Planned 2020 launch

ULTOMIRIS Subcutaneous

- Once-weekly, 10 min self-administration
- On-body commercially available device
- Planned 2022 launch

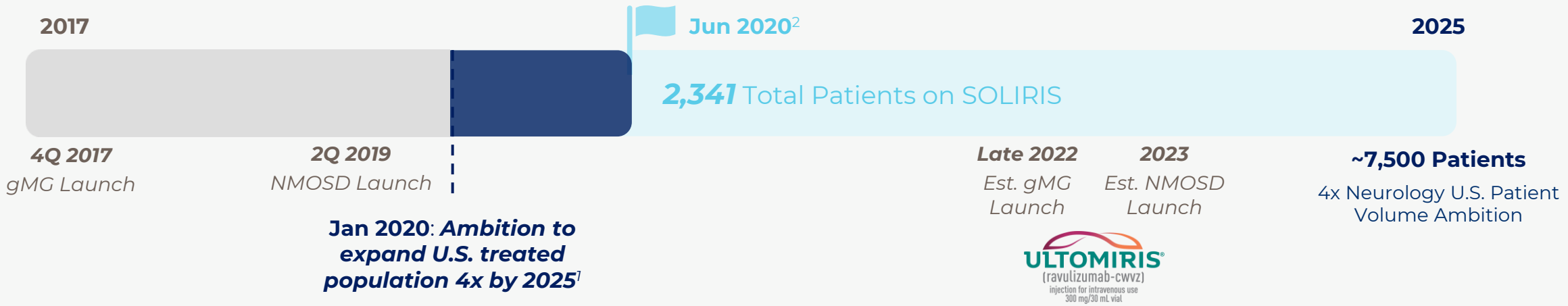
ALXN1720 Subcutaneous Mini-body

- Potential auto-injector or pre-filled syringe administration
- Plan to target broader rare diseases with burdensome , chronic standard of care

[1]US estimated launch; pending FDA approval; [2]On-going treatment cost after first year of treatment.

Neurology is Key Growth Driver through 2025

Significant Progress Thus Far Against 4X Neuro Ambition



OPPORTUNITY TO EXPAND NEUROLOGY PRESENCE WITH ULTOMIRIS IN GMG & NMOSD

[1]Ambition Baseline - 12/31/19 1,885 patients (4x growth ambition includes only gMG and NMOSD indications); [2]2,341 net patients on therapy as of Jun 30, 2020

ALS is a Devastating, Fatal Disease

ALS

- Disease Overview
- Mechanism of Action
- Clinical Development

Amyotrophic Lateral Sclerosis (ALS)

- Progressive loss of upper and lower motor neurons that inhibits signals
- Muscles begin to atrophy leading to loss of speech, paralysis, respiratory failure, and death
- Delayed time to diagnosis with average of 9-15 months from first symptom onset
- Average survival time is 3-5 years

Significant Unmet Need

- Available treatment options not effective
- Need for new treatment options with potential to halt or significantly slow disease progression and demonstrate survival and functional benefit
- Improved education and faster path to diagnosis

Estimated Addressable Population

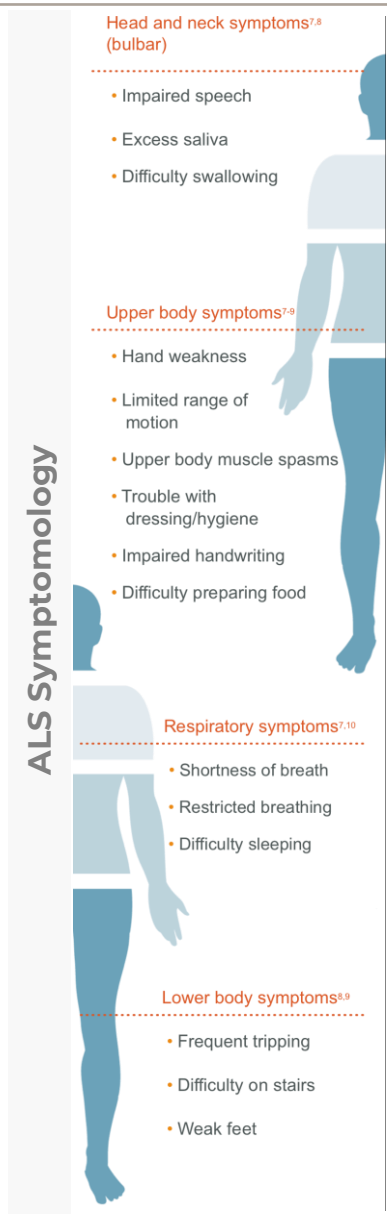
>40K
estimated diagnosed patients in US, EU, and JP

→

~75%
as 25% of patients may have late stage disease and may not benefit from treatment

→

~30K
addressable patient population with potential to benefit from ULTOMIRIS



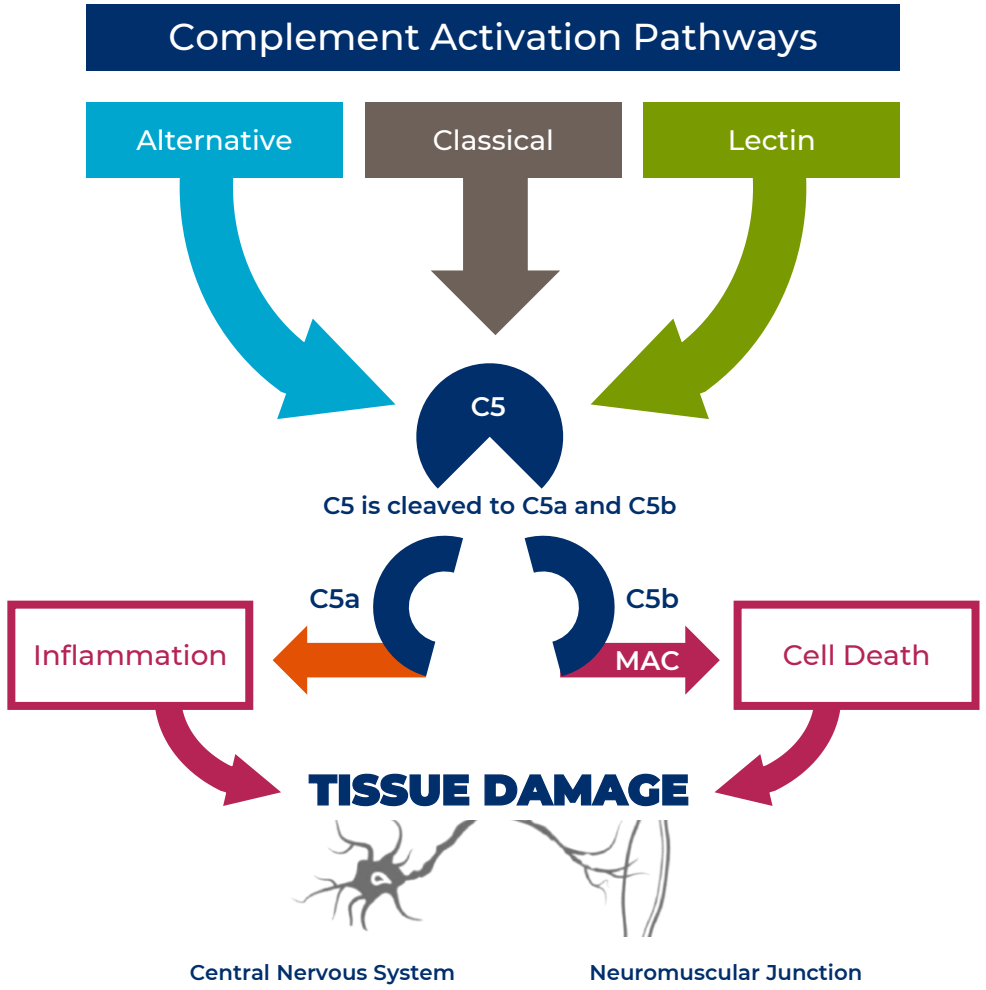
Role of Terminal Complement (C5) Inhibition in ALS

ALS

- Disease Overview
- Mechanism of Action
- Clinical Development

ULTOMIRIS Phase 3 ALS Trial Represents our Commitment to Pursue Promising Science

- Role of C5 inhibition in CNS and neuromuscular junction confirmed with SOLIRIS approval in gMG and NMOSD
- Preclinical evidence supports pursuit of ULTOMIRIS Phase 3 trial in ALS
  - Survival benefit shown in SOD1 mouse models through C5a inhibition or C5aR1 genetic knock-out
  - ALS patient tissue analysis shows elevated C5a levels in blood and lymphocytes; increased C5b-9 activity observed at neuromuscular junction prior to nerve death



COMPLEMENT IMPLICATED IN NEUROINFLAMMATION AND NEURONAL DEATH IN ALS

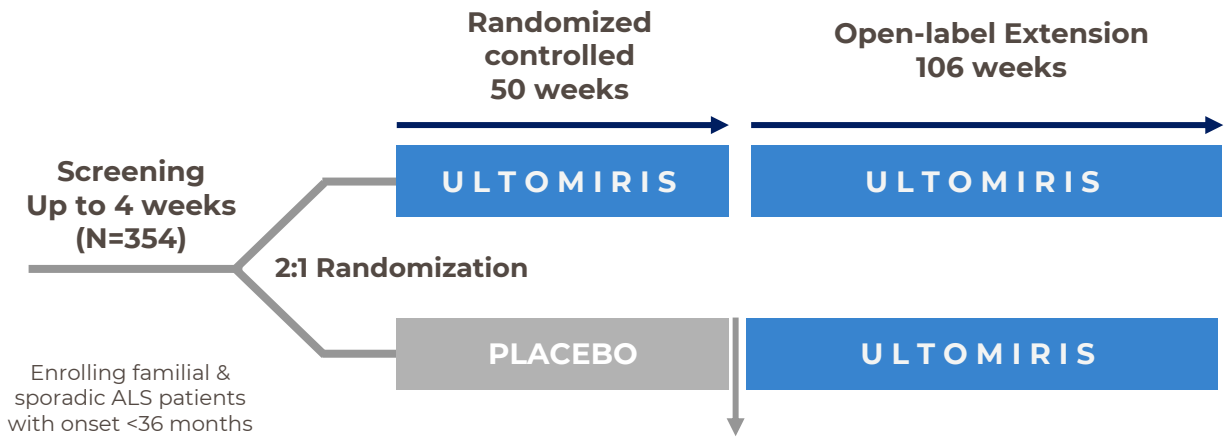
Sources: Woodruff TM, et al. *J Immunol 2008; (*181) 8727-8734 / Mantovani S, et al. *Journal of Neuroimmunology 2014*; (276) 213-218 / Parker SE, et al. *Neurobiology of Disease* 2019; (127) 223-232

ULTOMIRIS ALS Phase 3 Trial Design

ALS

- Disease Overview
- Mechanism of Action
- Clinical Development

Top-line Results Anticipated 2H 2022



Primary Endpoint: ALS-FRS-R

Key Secondary Endpoints: Ventilator-assisted free survival (VAFS), Slow Vital Capacity (SVC), Muscle strength (Hand-held dynamometry - HHD)

Potential ULTOMIRIS Value Proposition

- Potential to halt disease progression and symptom improvement
- Biomarker collection enhances evidence generation of role of C5 in ALS
- Development program built on patient and KOL insights which is critical in building a compelling value proposition

ULTOMIRIS HAS THE POTENTIAL TO BE FIRST-IN-CLASS C5 INHIBITOR IN ALS

ALXN1720: Internally Developed Third Generation C5 Inhibitor

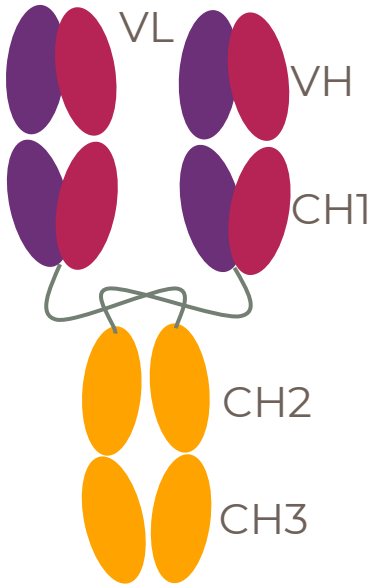
ALXN1720

Asset Overview

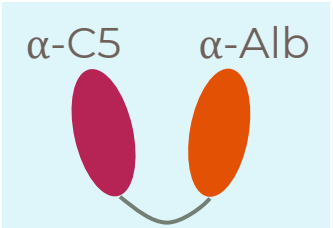
Indication Selection: gMG

Indication Selection: DM

ALXN1720: Bi-Specific Mini-body



eculizumab
(150 kDa)



ALXN1720
(25 kDa)

SMALL-VOLUME, ONCE-WEEKLY DOSING

Long-acting, small volume SC dosing

- **25 kDa molecular weight** (most antibodies ~150 kDa)
- **Potential for auto-injector or pre-filled syringe**
- **Extended half-life by binding to human serum albumin**

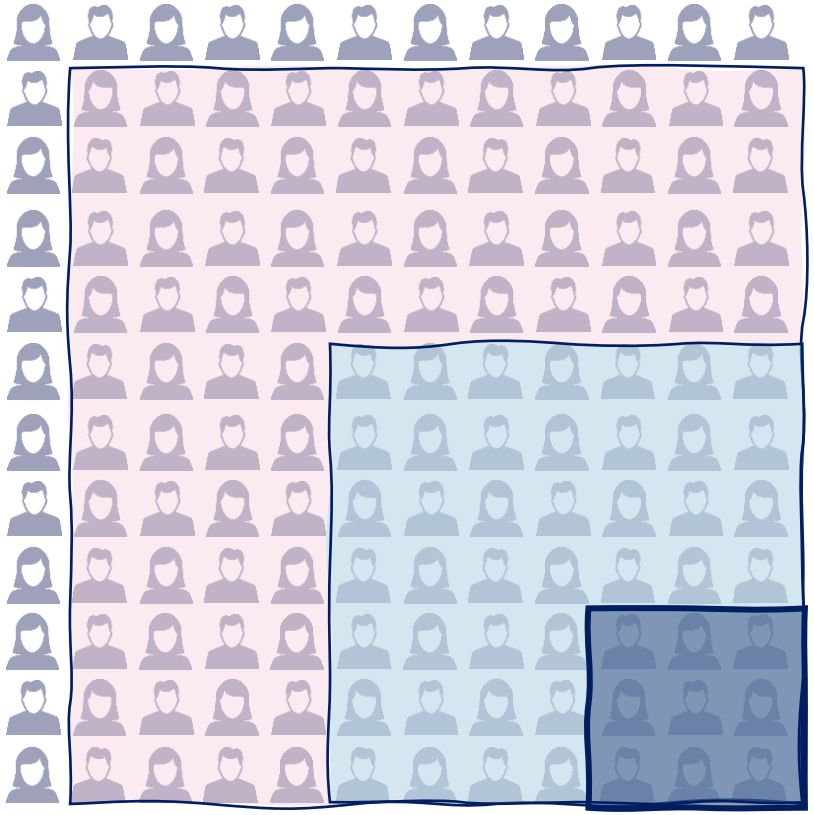
PHASE 1 TOPLINE DATA AND SUBSEQUENT PHASE 2 STUDY STARTS EXPECTED 1H 2021

Evolving our gMG Portfolio Strategy with ALXN1720

ALXN1720

- Asset Overview
- Indication Selection: gMG
- Indication Selection: DM

Expanding Addressable gMG Patient Population with ALXN1720



Ability with **ALXN1720** to treat earlier-line patients

- Mild, moderate & severe AChR+ patients
- Subcutaneous once-weekly dosing
- Auto-injector or pre-filled syringe
- Can co-administer with IVIg or anti-FcRn

ULTOMIRIS expands addressable patient population

- Moderate-to-severe AChR+ patients
- No prior treatment failure requirement
- ~30% discounted annual treatment cost per patient

SOLIRIS first approved gMG treatment in 60 years

- Refractory AChR+ patients who failed prior therapies

[1]AChR+: Acetylcholine receptor antibody-positive gMG represents ~85% of total gMG patients

Additional Neuromuscular Expansion in Dermatomyositis

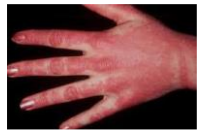
ALXN1720

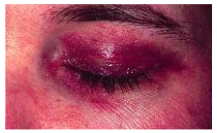
- Asset Overview
- Indication Selection: gMG
- Indication Selection: DM

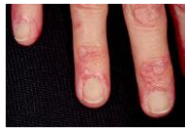
Dermatomyositis (DM) is a rare autoimmune inflammatory myopathy characterized by chronic inflammation and degeneration of muscle and skin

Classic DM: Muscular and Cutaneous Manifestations

Cutaneous: Visible Symptoms Often Support Diagnosis


Lesions on hands


Heliotrope rash


Gotton's papules

Muscular: Impact on Patient QoL Driven by Muscle Weakness

- Progressive muscle weakness that worsens over time
- Often involves hips, thighs, shoulders, upper arms, neck
- Everyday tasks (holding objects, rising from chair, climbing steps) are difficult
- Difficulty swallowing, breathing problems
- Despite therapy, ~1/3 of patients are left with mild to severe disability

- **With SoC therapy, 5-year mortality is ~25%**
- **Patients' cancer risk is elevated**

Role of Complement in DM

- Classical and alternative pathway proteins are upregulated in muscle of dermatomyositis (DM) patients[1]
- Terminal complement (C5b-9) deposition on non-necrotic and necrotic muscle fibers[2]
- Eculizumab improved clinical condition and biological parameters in a juvenile patient at 900 mg QWx5 - >1200mg Q2W[3]

DM Diagnosed Prevalence
~50K
Classic DM Diagnosed Prevalence
~40K

DEVELOPMENT PROGRAM DESIGN UNDERWAY WITH PLANS TO INITIATE IN 2021

[1]Nagaraju, et al. 2016; [2]Wang 2018; [3]Vanoni 2014

Q&A Session I

30 minutes

ALXN1840 in Wilson Disease

John Orloff, M.D.
Head of R&D

Brian Goff
Chief Commercial & Global Operations Officer

Wilson Disease is a Rare Inherited Disorder

Wilson Disease

Disease Overview

Patient Journey

Mechanism of Action

Clinical Development

Path to Market

- Devastating disorder characterized by copper accumulation in the liver, brain, and other organs
- If left untreated, risk of eventual cirrhosis or liver failure
- Need unaddressed by current chelators:
  - De-coppering and elimination through normal excretion
  - Poor compliance
  - Potential for neurological worsening

Diagnosed Prevalence

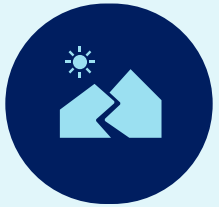
 **~5K**

 **~5K**

 **~2.5K**

Genetic prevalence believed to be higher and potential to improve for some patients with increased awareness and new diagnostic practices

Significant Unmet Need



Challenging Path To Diagnosis

- Heterogeneous symptomology
- Complex diagnostic algorithm
- High rate of mis-diagnosis

Poor Compliance Rate with Standard of Care (SoC)

- Current SoC consists of cycles of de-coppering agents followed by zinc maintenance
- Burdensome treatment regimen and side-effects
  - 2-4x daily oral requiring 6-12 hrs fasting per day

Neurological Worsening

- Long-term neurological effects include impaired motor skills and psychiatric changes (behavior and personality)
- Current SoC does not address neurological impacts of disorder

Our Patients are Our Inspiration: Meet Cory

Wilson Disease

- Disease Overview
- Patient Journey
- Mechanism of Action
- Clinical Development
- Path to Market

Cory living with Wilson Disease

ALXN1840: Novel Oral Potential Treatment for Wilson Disease

Wilson Disease

Disease Overview

Patient Journey

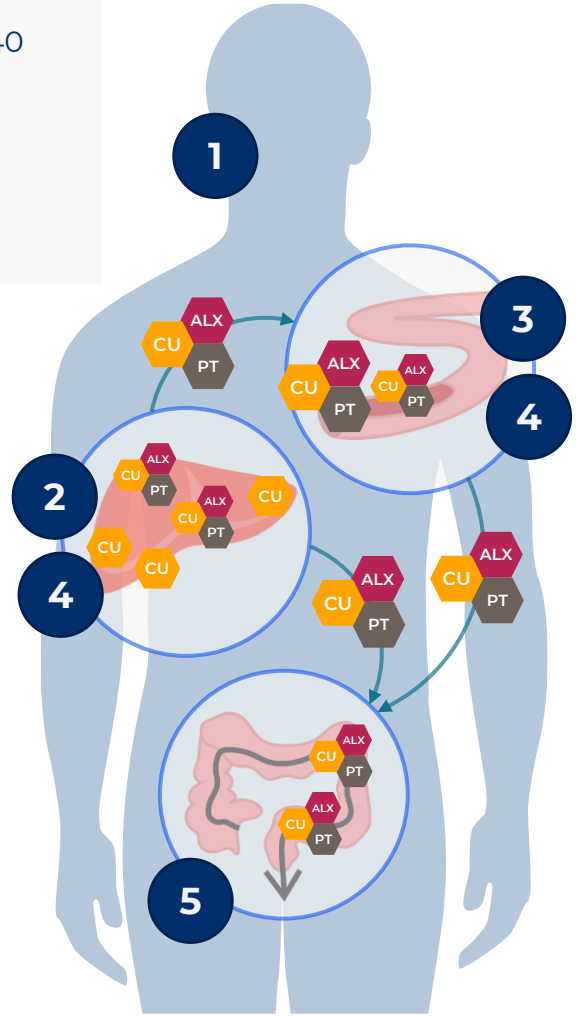
Mechanism of Action

Clinical Development

Path to Market

Tripartite Complex

- ALX — ALXN1840
- PT — Protein
- CU — Copper



1. ALXN1840 dosing regimen: ***Oral, once-daily dose***[1,2]
2. High affinity to copper (10,000x): direct binding and **removal of copper from intracellular stores** in the liver[3]
3. ALXN 1840 **specifically binds to copper**[1]
4. ALXN1840 + copper forms stable **tripartite complex** with proteins[1,2]
5. **Normal biliary excretion** of copper[2]

1. Weiss KH et al. *Lancet Gastroenterol Hepatol* 2017;2:869-876; 2. Stremmel W. *Hepatology* 2019;69:901-903; 3. Plitz T and Boyling L. *Xenobiotica* 2018;49:332-338

New Biomarker May Change Wilson Disease Diagnostic Paradigm

Wilson Disease
Disease Overview
Patient Journey
Mechanism of Action
Clinical Development
Path to Market

Alexion is developing a new biomarker designed to directly quantify the **labile bound copper (LBC)** fraction

LBC assay for screening, diagnosing, and assessing copper control in patients

Expected to allow clinicians to easily and accurately assess patients

Establish reliable treatment targets that directly align with ALXN 1840 treatment goals

Suitable for the analysis of all pre- and post-dose samples, regardless of treatment

NEW ASSAY TO SUPPLEMENT WILSON DISEASE AWARENESS EFFORTS

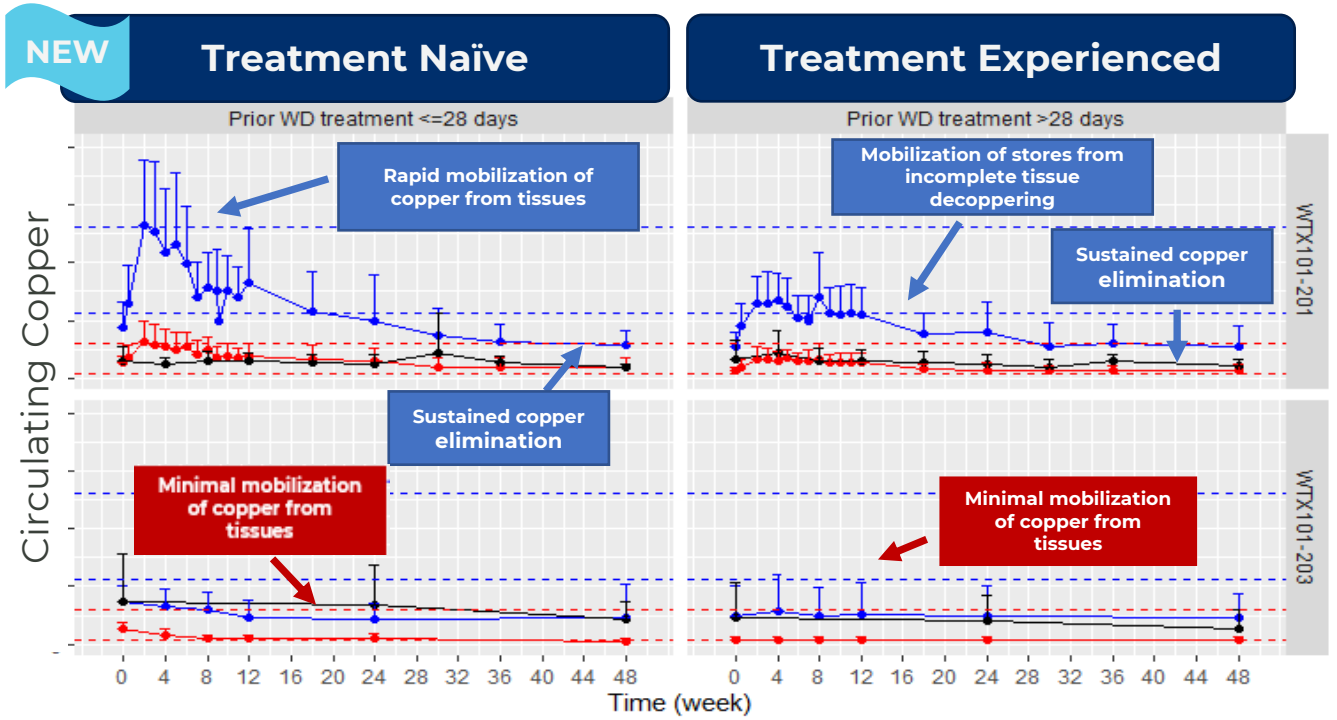
New Phase 2 Data Suggests Potential To Redefine Treatment Goals

Wilson Disease

- Disease Overview
- Patient Journey
- Mechanism of Action
- Clinical Development
- Path to Market

Recent Phase II data shows that ALXN1840 actively and dynamically mobilizes Cu from tissue stores

NEW



ALXN1840

Circulating Copper

Urine Copper

Standard of Care

Based Upon Ph2 Data, Exploring A Revised Endpoint To Support:

Superiority of ALXN1840 over SOC

Potential paradigm shift in treatment from plasma copper guided to whole body copper mobilization and excretion approach

Global access & value strategy

Additional Programs Designed to Support Broader Decoppering:

Copper Balance Program

- Measure copper mobilization & elimination through biliary system

Liver Biopsy Program

- Measure liver tissue de-coppering

Commercial Plan to Bring ALXN1840 to Patients

Wilson Disease

- Disease Overview
- Patient Journey
- Mechanism of Action
- Clinical Development
- Path to Market

Key Objectives For Launch

1. **Drive Education, Awareness & Diagnosis**
   - Increase awareness amongst key physicians
   - Develop early screening tools
   - Introduce LBC Assay to improve screening, diagnosis, and assessment of copper control in patients
   - Increase awareness that early diagnosis/treatment can prevent often irreversible psychiatric impacts
2. **Revolutionize the Treatment Paradigm**
   - ALXN1840 is a first-in-class agent that binds copper with high affinity and specificity
   - ALXN1840 has a unique mechanism (proven in Ph2) for copper control
     - Rapid, sustained control of copper and clinical symptoms with low risk of neurological worsening
3. **Drive Access & Conversion from Current Treatments**
   - Leverage potential strength in clinical profile and convenience of once-daily oral administration
   - Mobilize health economics and access resources to support labeling, access, and pricing focused on superiority (based on potential Ph3 results)

Development Progress Continues

2018
Alexion Acquires Wilson Therapeutics and Re-Powers Ph3 Study for Superiority vs. SoC

1Q2020
Ph3 FoCuS Trial Enrollment Completion

2Q2021
Top Line Results

3Q2021
US Filing

2H2022
US Launch

CAEL-101 in AL Amyloidosis

Cristina Quarta, M.D., Ph.D.
CAEL-101 Clinical Development Lead

- More than 15 years of experience specializing in field of systemic amyloidosis in major international amyloid centers
- Published over 50 manuscripts including global guidelines on diagnosis and management of cardiac amyloidosis
- Joined Alexion in 2019

AL-Amyloidosis: Hematological, Severe, Multi-Organ Disorder

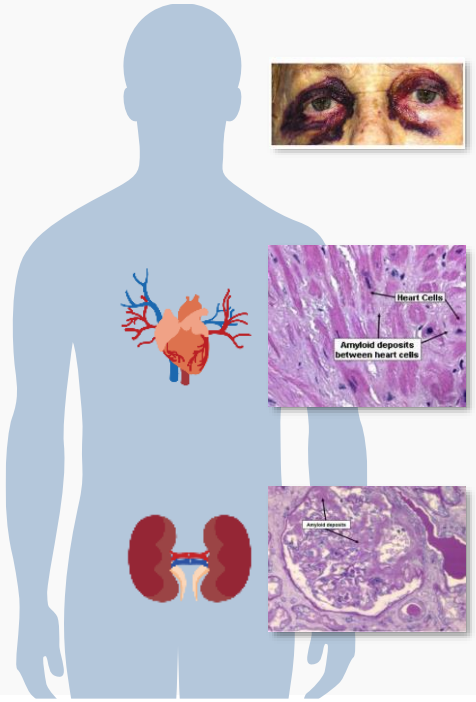
AL-Amyloidosis

- Disease Overview
- Clinical Data
- Clinical Development
- Potential New SoC

A **plasma-cell dyscrasia** characterized by an autonomous proliferation of plasma cells with an **overproduction** of a monoclonal **IgG**

Severe **multi-organ damage** most frequently **heart, kidneys, and peripheral nerves**

Difficult to recognize because of its broad range of manifestations and vague symptoms, **and lack of awareness**



Median Survival <18 Months from Diagnosis

Primary Treatment Goals

1. Suppression of amyloid protein precursors
2. Reduction of amyloid deposits in the organs
3. Symptom relief, survival & organ function improvement

Unmet Medical Need

~20K
US + EU Addressable Population
(Mayo Stage IIIa + b)

Early Data Supports Role of CAEL-101 in AL-Amyloidosis

AL-Amyloidosis

- Disease Overview
- Clinical Data
- Clinical Development
- Potential New SoC

CAELUM BIOSCIENCES

CAEL-101 is a chimeric mAb with high specificity to kappa and lambda light chain antibodies

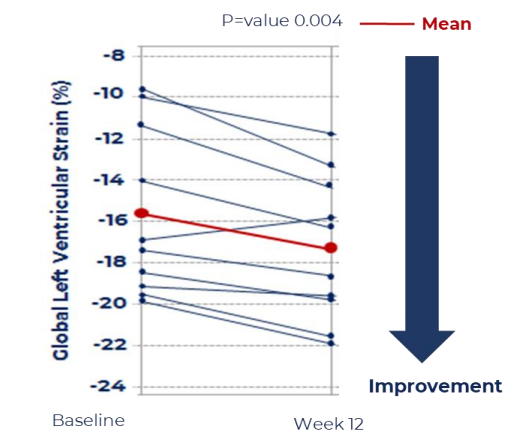
Phase 1a/1b trial was first in AL-Amyloidosis to show improvement in cardiac function via GLS

Global Longitudinal Score (GLS) is a better predictor of survival vs. cardiac biomarkers

Long-term Phase 1a/1b data show 78% survival (15/19) at median follow-up of more than 3 years (37 months) in patients treated with CAEL-101

Left ventricular data:
Phase 1b Cardiac Patients (N=10)

Mean absolute improvement of 1.69 points in GLS score after 12 weeks of treatment



- Evidence suggests patients treated with chemotherapeutic PCD agents showed no change in GLS from baseline to at least 1 year
- **9 of 10 cardiac patients improved compared to baseline**
- **CAEL-101 was well-tolerated, no dose-limiting toxicity or drug-related deaths; maximum tolerated dose 500mg/mg²**

Edwards CV, et al. Final Analysis of the Phase 1a/b Study of Fibril-Reactive Monoclonal Antibody 11-1F4 (CAEL-101) in Patients with AL Amyloidosis
Bhutani, et al. Long term follow-up of patients with AL Amyloidosis treated on a phase 1 study of Anti-Amyloid Monoclonal Antibody CAEL-101

Transforming AL-Amyloidosis Treatment Landscape

AL-Amyloidosis

- Disease Overview
- Clinical Data
- Clinical Development
- Potential New SoC

CAEL-101 Phase 2/3 Program Design

Ph2 Dose Selection ✓

↓

Ph3 Twin RCT

Event Driven Trial (Minimum of 50 Weeks Tx)

n = 267	*n = 111*
Mayo Stage IIIa	Mayo Stage IIIb
Occurrence of 77 Deaths	Occurrence of 54 Deaths

QW IV infusions for 4 weeks, then Q2W

Primary Endpoint: Overall Survival
Secondary Endpoints: Patient Function (6MWT), QoL (KCCQ-OS and SF-36v2 PCS), and Cardiac Imaging (GLS%)

CAEL-101 to be studied alongside plasma cell dyscrasia (PCD) treatment

CAEL-101 has potential to remove amyloid deposits from tissues and improve organ function, with aim of prolonging survival

With Potential to Change Standard of Care

Current	Future
Amyloid Suppression: ---------- **CyBorD**	**Amyloid Suppression:** **CyBorD + Daratumumab** + **Amyloid Removal:** **CAEL-101**

ON TRACK FOR POTENTIAL LAUNCH BY 2023; OPTION TO ACQUIRE CAELUM POST PHASE 3 DATA

Factor D Platform

Gianluca Pirozzi, M.D., Ph.D.
Head of Clinical Development and Translational Sciences

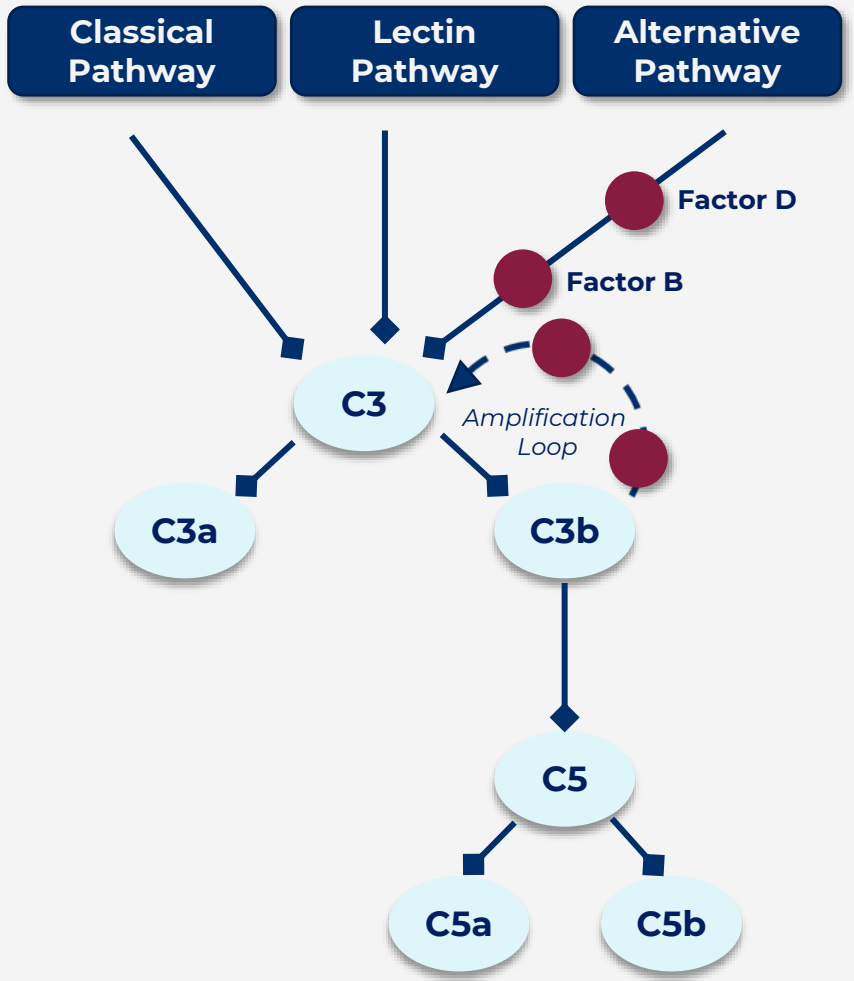
- Over two decades of drug development experience
- Previously served as a Board Member of Imbria Pharmaceuticals and current Board Member of Timber Pharmaceuticals
- Scientific Advisor for the Smith-Magenis-Syndrome Research Foundation
- Joined Alexion in 2019

Targeting Factor D to Inhibit Alternative Pathway

Factor D

- Mechanism Overview
- Therapeutic Application
- Evolving in PNH
- PNH Clinical Development
- GA Overview
- Role of Complement in GA
- GA Clinical Development
- Factor D in Renal

Complement Cascade



- Factor D is a key, rate-limiting enzyme of the complement alternative pathway
- Selective inhibition of the alternative pathway keeps classical and lectin pathways intact to fight infections
- Proof of Concept shown for Factor D inhibition in multiple complement-mediated rare diseases
- More likely to maintain consistent control than drugs targeting C3 or Factor B
  - C3 and fB are acute phase reactants (increasing levels during inflammation or stress)

Factor D an Attractive Target for AP Inhibition

More tractable therapeutic target vs Factor B given much lower concentration in blood

Factor D Platform

Factor D

- Mechanism Overview
- Therapeutic Application
- Evolving in PNH
- PNH Clinical Development
- GA Overview
- Role of Complement in GA
- GA Clinical Development
- Factor D in Renal

Two Unique Assets Targeting Factor D

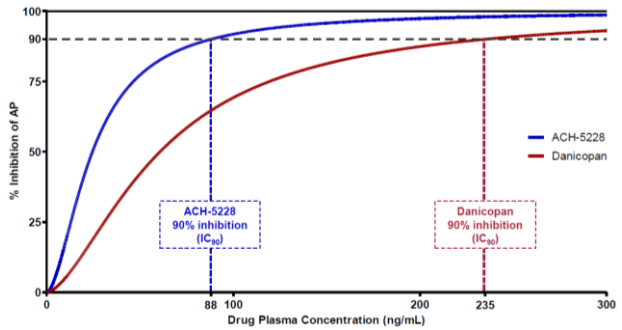
ALXN2040

- Optimal biodistribution in the retina, supports potential use in ophthalmology indications

ALXN2050

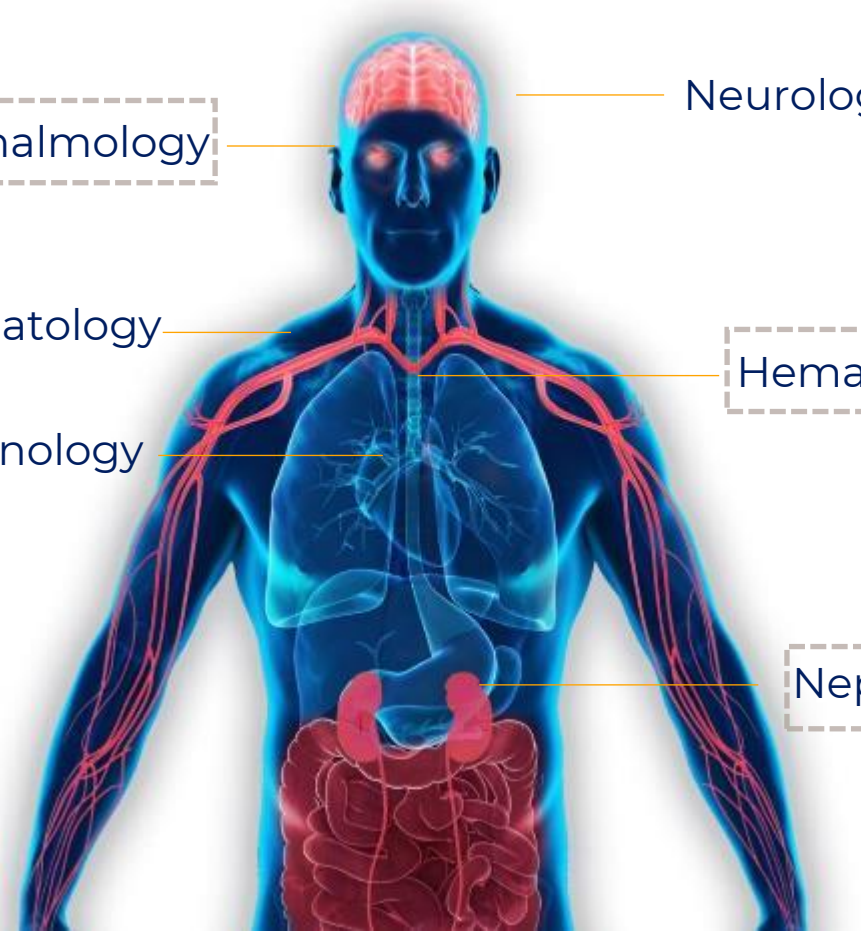
- Longer half-life and more potent alternative pathway inhibition supports potential use in PNH monotherapy and renal disease

Multiple Ascending Dose Results



- AP = alternative pathway; danicopan = ALXN2040; ACH-5528 = ALXN2050
- >95% AP inhibition at mean steady state trough concentrations in P1 MAD study in healthy volunteers as measured by AP Hemolysis and AP Wieslab assays

Wide Range of Therapeutic Areas of Interest



Anita Hill, M.D., Ph.D.
Hematology Global Medical Affairs Lead

- Prior Lead Clinician for the National PNH Service, UK and Honorary Associate Professor for the University of Leeds
- Managed over 500 patients with PNH in her career
- Joined Alexion in February 2020

Darius Moshfeghi, M.D
Professor, Stanford University & GA Program Clinical Consultant

- Professor of Ophthalmology, Stanford University School of Medicine
- Chief of Retina Division, Byers Eye Institute
- Joined Alexion in 2020 as clinical consultant for GA

Alexion Continues to Innovate for Patients with PNH

Factor D

Mechanism Overview

Therapeutic Application

Evolving in PNH

PNH Clinical Development

GA Overview

Role of Complement in GA

GA Clinical Development

Factor D in Renal

Before — *Introduction of C5 Inhibitor Therapy* — *Future*

SOLIRIS® (eculizumab) Injection for Intravenous Use 300 mg/30 mL vial

ULTOMIRIS® (ravulizumab-cwvz) injection for intravenous use 300 mg/30 mL vial

Patients managed with supportive care

20-35% died within 6 years of diagnosis

First approved treatment in PNH

Improved overall survival to similar level of general population

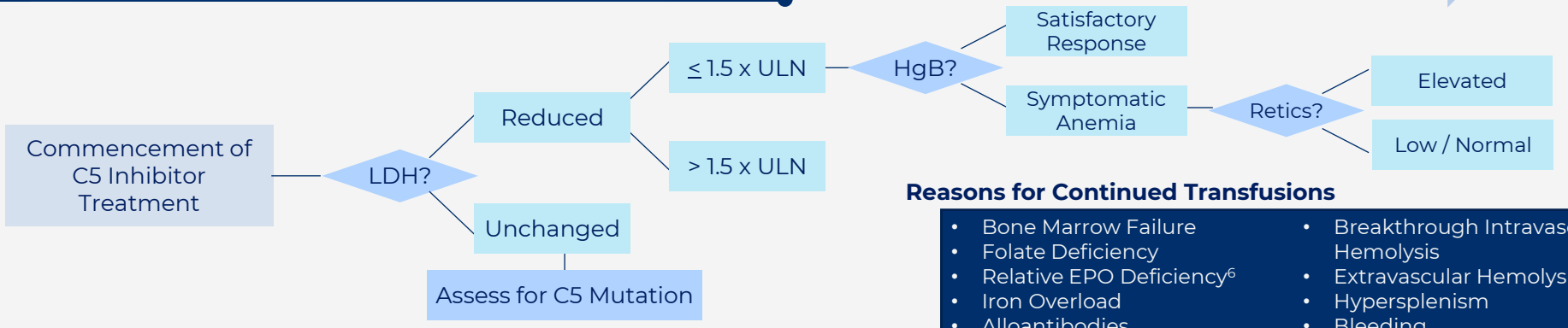
First and only long-acting C5 inhibitor

Maximal IVH control and reduction in BTH[1], while reducing treatment burden

Enhancing Treatment Offering

Transfusion dependent EVH[2] and additional patient choice to further reduce treatment burden

Management and Care of PNH Patients in Practice



Reasons for Continued Transfusions

- Bone Marrow Failure
- Folate Deficiency
- Relative EPO Deficiency[6]
- Iron Overload
- Alloantibodies
- Breakthrough Intravascular Hemolysis
- Extravascular Hemolysis
- Hypersplenism
- Bleeding

~10% OF PNH PATIENTS SUFFER FROM TRANSFUSION DEPENDENT EVH

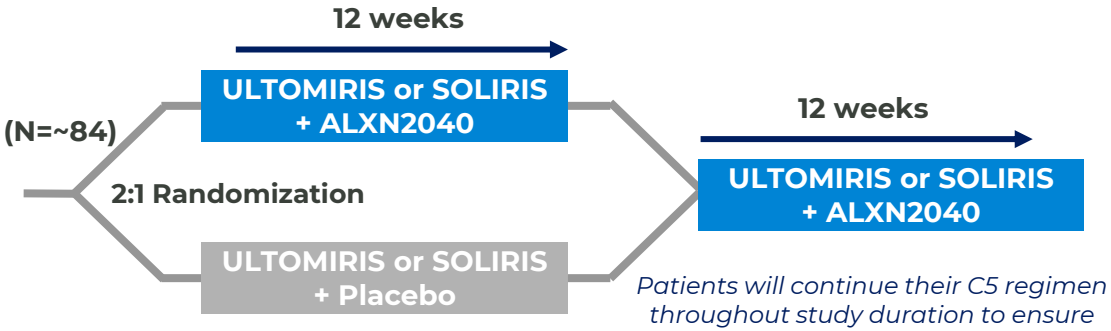
[1]BTH: breakthrough intravascular hemolysis; [2]EVH: Extravascular hemolysis; [3]HgB: Hemoglobin; [4]Retics: Reticulocytes; [5]ULN: Upper limit of normal; [6]EPO: Erythropoietin

Progressing Factor D Development Plans in PNH

Factor D

- Mechanism Overview
- Therapeutic Application
- Evolving in PNH
- PNH Clinical Development
- GA Overview
- Role of Complement in GA
- GA Clinical Development
- Factor D in Renal

Phase 3 ALXN2040 Combination Study

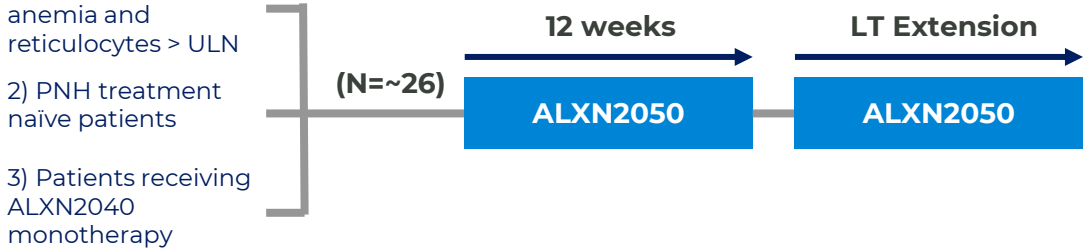


Patients will continue their C5 regimen throughout study duration to ensure continued IVH control

Study to Initiate 4Q 2020

TLR Estimated 2H 2022

Phase 2 ALXN2050 Study



Study On-Going

TLR Estimated 2H 2021

Targeting Factor D In PNH

ALXN2040:
Combination Therapy To Address EVH

- Potential to address clinically evident EVH for patients on ULTOMIRIS or SOLIRIS (<10% of patients) with 2040
- Primary Endpoint: Change from Baseline in hemoglobin

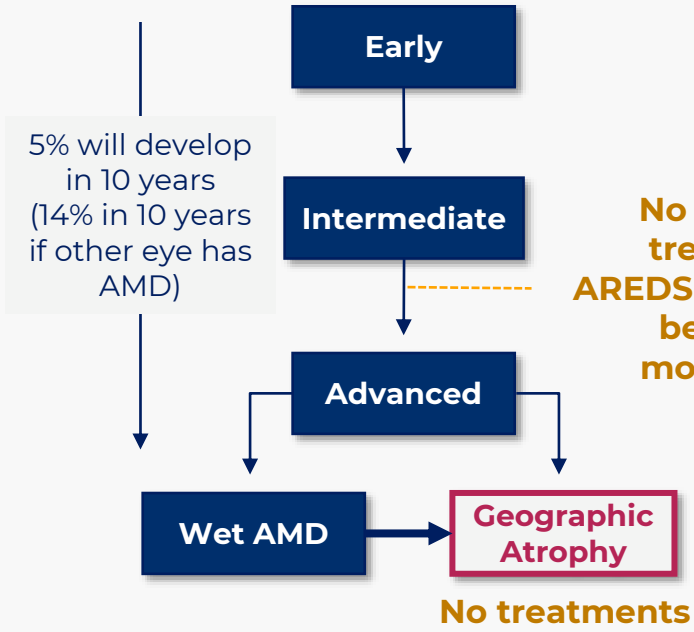
ALXN2050:
Potential Transformational Oral Therapy

- Ability to build on fD platform through 2050 and address IVH with no C3 deposition leading to EVH
- Continue to ease treatment burden on patients & healthcare system through an oral administration only
- Reduce burden on healthcare systems & resources

Geographic Atrophy & Broader AMD Landscape

Factor D
Mechanism Overview
Therapeutic Application
Evolving in PNH
PNH Clinical Development
GA Overview
Role of Complement in GA
GA Clinical Development
Factor D in Renal

Age-Related Macular Degeneration (AMD)



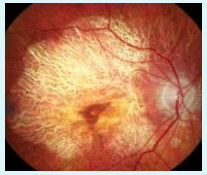
Non-Central GA

(does not involve fovea)

- 80% to 85% of patients at initial diagnosis
- Difficult to detect if other eye has normal vision

Central GA

(involves fovea)



- 15% to 20% of patients at initial diagnosis
- Patients may only retain eccentric gaze

Geographic Atrophy (GA)

- Chronic and progressive degeneration of the portion of the retina responsible for central & color vision
- Slow rate of progression and subtle symptoms leads to lower rates of diagnosis than wet AMD
  - One affected eye typically impacted first
- Disproportionately affects those 75 and older
- Affects >2M patients across US and EU
  - 40% of whom are diagnosed and addressable
  - Growing 3-5% per year as population ages

High Disease Burden for Patients

Major cause of blindness

High levels of visual impairment and loss of mobility

Significant reductions in quality of life

Role of Complement and AP Inhibition in Geographic Atrophy

Factor D

Mechanism Overview

Therapeutic Application

Evolving in PNH

PNH Clinical Development

GA Overview

Role of Complement in GA

GA Clinical Development

Factor D in Renal

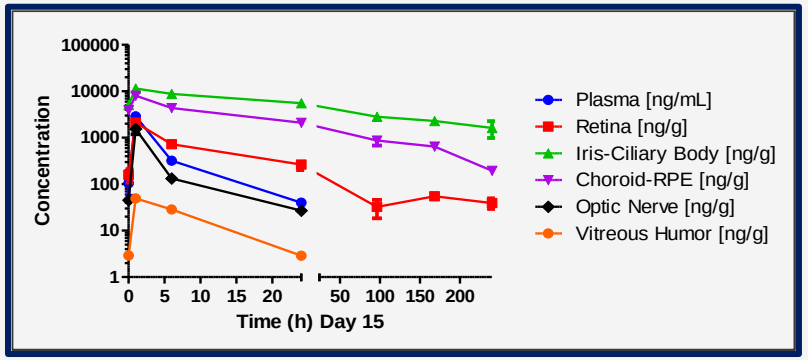
Complement Inhibition Proven Role In GA

- Human studies demonstrate evidence of AP complement activation
  - Complement byproducts in donor eye and plasma/serum samples
- Studies show complement genetic factors implicated in risk factors for AMD, including Factor D
- Intravitreally injected complement inhibitors have demonstrated PoC in Phase 2 studies

ALXN2040 Preclinical Data

Two unique traits of ALXN2040 lead to higher and sustained exposure of the drug in choroid, RPE and retina after oral dosing

- Binds to melanin
- Able to pass blood-retina-barrier
- Potential QD dosing



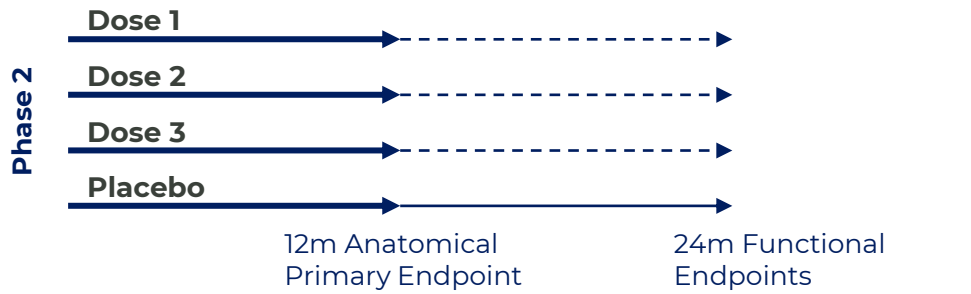
Promising ALXN2040 PK data in Dutch belted rabbit model

ALXN2040 in Geographic Atrophy

Factor D

- Mechanism Overview
- Therapeutic Application
- Evolving in PNH
- PNH Clinical Development
- GA Overview
- Role of Complement in GA
- GA Clinical Development
- Factor D in Renal

Phase 2 ALXN2040 in GA



Assessing dosing with potential for once or twice daily regimen

Note actual number of doses in Phase 2 may vary

Move to Phase 3

Study to Initiate 2H 2021

Aim To Reduce Disease Progression And Preserve Vision In Patients At Risk For Permanent Vision Loss

- Oral dosing vs intravitreal injections
  - Leading to improved patient acceptance
  - Systemic approach with advantage of treating both eyes simultaneously
- Improve QoL for patients and ability to perform activities of daily living and maintain independence
- Delivers opportunity to intervene earlier in disease for some patients
- Reduce secondary complications due to GA
- Potential for significant health economic advantages versus intravitreally injected therapies

ALXN2040 is uniquely positioned to address the unmet need in GA based on its ability to cross the retina-blood-barrier and its sequestration in ophthalmic tissue

Alternative Pathway Inhibition in Rare Renal Diseases

Factor D

- Mechanism Overview
- Therapeutic Application
- Evolving in PNH
- PNH Clinical Development
- GA Overview
- Role of Complement in GA
- GA Clinical Development
- Factor D in Renal

The Alternative Pathway (AP) Is A Strong Mediator Of Abnormal Immune Response Across Several Renal Diseases

- Alternative Complement Pathway activation drives inflammation and an aberrant immune response in the kidney
- Complement target therapy has potential to treat underlying pathophysiology and improve clinical outcomes
- Goal to reduce occurrence end stage renal disease, need for transplant or dialysis, and mortality

Sampling of Potential Basket Trial Indications

Lupus Nephritis (LN)	IgA Nephropathy (IgAN)	Primary Membranous Nephropathy (PMN)	Complement 3 Glomerulopathy (C3G)
~60K US Diagnosed Prevalence	~90K US Diagnosed Prevalence	~80K US Diagnosed Prevalence	<6K US Diagnosed Prevalence
• Despite treatment, up to ~30% develop end stage renal disease under current SOC	• Approx. 40% of IgAN patients progress to end stage real disease • Greatest unmet need is control of proteinuria	• 10-20% of patients progress to end stage renal disease under current SOC	• Median time to end stage renal disease is ~10 years • In pts who receive kidney transplant, ~70% experience relapse

Planned Ph2 PoC Trials 1H2021

Significant Potential Value Exists In Nephrology

- High unmet need, particularly in more severe or refractory populations
- High enthusiasm for novel MOAs and approaches; treatments with noteworthy incremental benefit
- Recent developments in the regulatory environment have clarified and expedited development pathways
- POC/Basket approach with Factor D and C5 de-risks the portfolio and allows for nimble decision making as development progresses and treatment landscape evolves

POTENTIAL PREVALENCE OF >200K PATIENTS; ENORMOUS UNMET MEDICAL NEED

Anti-FcRn Platform

Gianluca Pirozzi, M.D., Ph.D.
Head of Clinical Development and Translational Sciences

Anti-FcRn: Targeting Numerous IgG-Mediated Diseases

ALXN1830

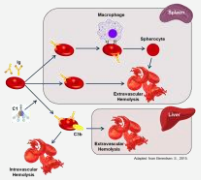
FcRn Landscape

Clinical Development

- **Broad applicability** across numerous rare IgG-mediated autoimmune diseases
- **Product differentiation** expected to come from efficacy, IgG lowering effect, and convenience of administration
- **Long-term safety profile** to be demonstrated with perceived differences across assets (albumin reductions & headaches)

Warm Autoimmune Hemolytic Anemia (WAIHA)

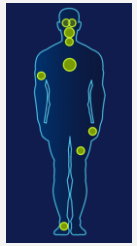
Rare disease driven by presence of pathogenic IgG autoantibodies that trigger hemolysis at normal body temperature



- Patients face extreme weakness and fatigue; Inherent risk of thrombotic events
- Currently no approved therapies. Most common treatments include steroids, rituximab, splenectomy
- ~65K diagnosed prevalent patients in the US and EU

Generalized Myasthenia Gravis (gMG)

Rare chronic, autoimmune, neuromuscular disease that can cause progressive muscle weakness and disability



- Patients face drooping eyelids, blurry or double vision, slurred speech, difficulty swallowing or choking, shortness of breadth, weakness in limbs that can be debilitating
- Expanding Alexion's portfolio of treatment options for gMG patients in need
- ~60-80K total gMG patients in the US; targeting mild to moderate patients

Pursuing Path Forward with Subcutaneous Formulation

ALXN1830

FcRn Landscape

Clinical Development

Positive Early Signal from SC Phase 1 Study

SC single doses suggest meaningful IgG-lowering potential prior to study pause due to COVID-19

- Preliminary PK/PD modeling suggests 1500mg weekly SC may have the potential to provide **>70% IgG lowering**
- Dosing would be compatible with **convenient SC delivery via on-body device**

HV Ph1	1H 2021	2H 2021
ALXN1830 SC **WAIHA**	SAD/MAD	
ALXN1830 SC **gMG**		Ph2
ALXN1830 SC		Ph2

ALXN1830 Value Proposition

- Superior dosing profile with once weekly subcutaneous administration
- High specificity to IgG
- No effect on albumin, eliminating safety concerns of hypoalbuminemia; no headache seen thus far in SC HV
- Rapid onset of action and sustained IgG lowering after a single dose

PLAN TO RE-INITIATE SC FORMULATION DEVELOPMENT PLANS EARLY IN 2021

Internal Research & Discovery

Sharon Barr, Ph.D.
Head of Research,
Bioinformatics & Diagnostics

- Responsible for drug discovery and early development
- Over 15 years of industry experience in precision medicine
- Serves on the board of the Alexion Charitable Foundation
- Joined Alexion in 2013

Enhancing our Foundational Complement Research Platform

Building New Capabilities

Collaborations, Partnerships & Acquisitions

- fD small molecule engineering (Achillion)
- C6 Targets (Complement Pharma)
- RNAi Technology (Dicerna)
- Peptide technology (Zealand)

Complement Research & Discovery

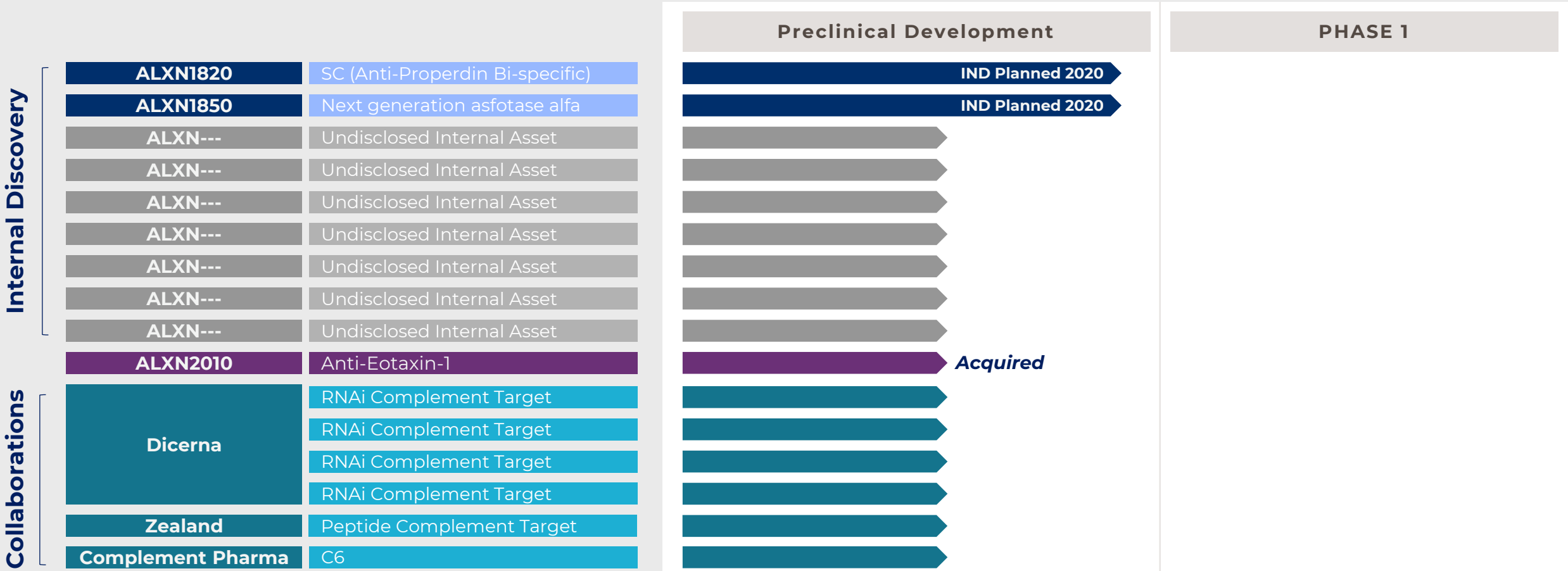
Expertise in Ab Engineering

C5-targeted Continuous Innovation

- ULTOMIRIS Q8W mAb IV / SC
- ALXN1720 Bi-specific Mini-body
- ALXN1820 Bi-specific Anti-properdin

AMBITION TO FILE >5 NOVEL INDS BY 2025

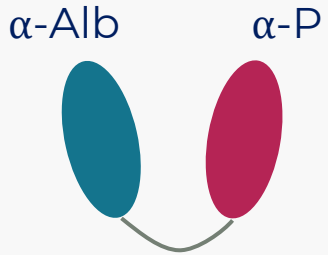
Robust Early Stage Pipeline



STATE OF THE ART ANTIBODY ENGINEERING PLATFORM & NEW TECHNOLOGY COLLABORATIONS

Internally Designed Novel Assets: ALXN1820 & ALXN1850

ALXN1820 – Bispecific anti-properdin



- Blocks properdin, binds to albumin, extending half life
  - Anti-properdin mechanism of action has shown high C3 inhibition without affecting bacterial opsonization (lower infection risk)
- In-vivo studies indicate well-tolerated safety
- Weekly self-administered subcutaneous dosing
- Potential therapeutic area applications include hematology, pulmonology, nephrology, and dermatology

ALXN1850 – Next generation asfotase alfa

- Next generation alkaline phosphatase enzyme replacement therapy for hypophosphatasia
  - Engineered catalytic domain has higher activity vs. natural substrates than asfotase alfa
- In-vivo studies demonstrate potential for enhanced efficacy and well-tolerated safety; high in-vivo exposure
- Projected in-human dose significant reduction from current STRENSIQ dose and number of weekly doses
  - Suitable for weekly self-administrated subcutaneous dosing
- Potential for expansion beyond pediatric onset HPP

PLANNED IND FOR BOTH 1820 AND 1850 IN THE SECOND HALF OF 2020

Q&A Session II

30 minutes

Rachel living with gMG

CEO Concluding Remarks

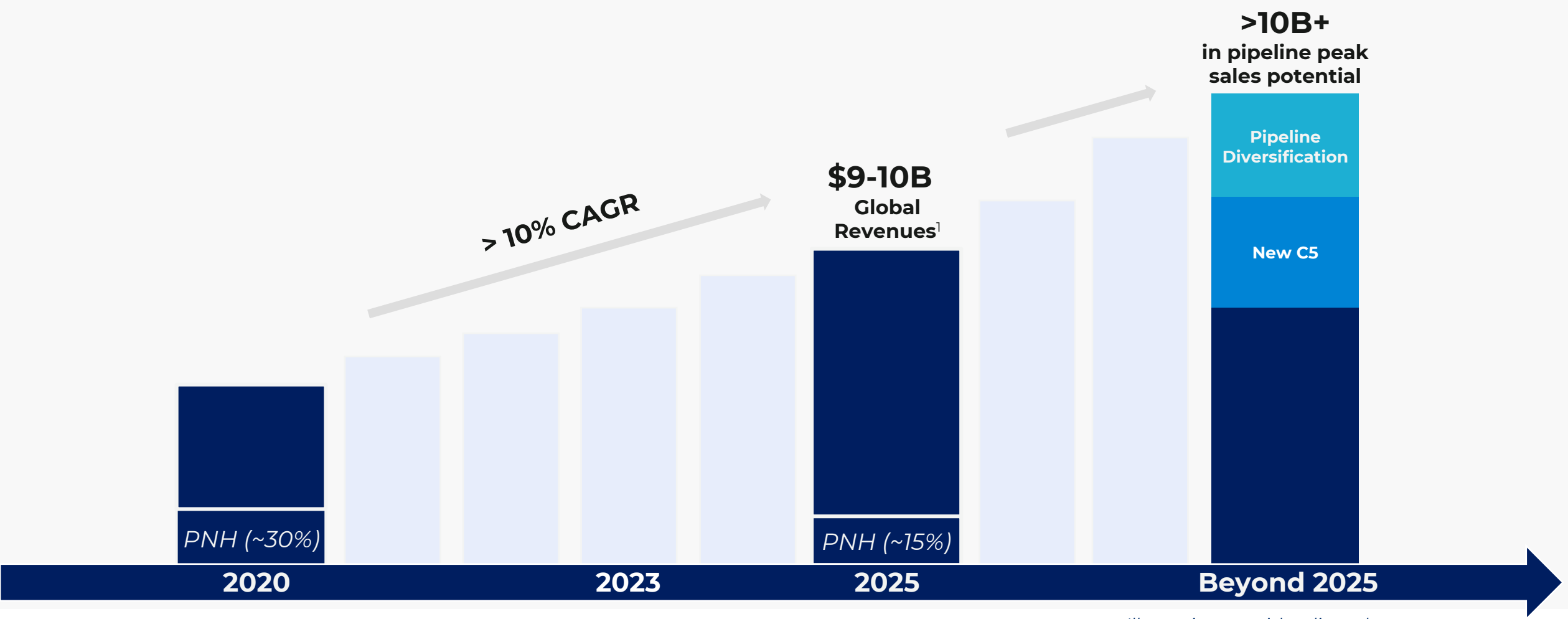
Ludwig Hantson, Ph.D.
Chief Executive Officer

Key Takeaways

- Sustainable growth in portfolio **targeting $9-10B in revenue in 2025**[1] (current consensus estimate ~$7.5B[2]) while maintaining >50% non-GAAP operating margins
- **Current pipeline contributes >$10B in peak sales potential beyond 2025**
- Differentiated as a **biotech focused in rare disease** with resourcing capacity to reinvest in our pipeline and commercial capabilities; ambition for >5 INDs by 2025
- Robust pipeline with **terminal complement, Factor D and anti-FcRn Platforms** and **novel rare disease assets** contributing to 7 blockbuster franchises; multiple pivotal results in next 12+ months

[1] 2025 $9-10B target is at constant currencies (9/30/20 levels); [2] Consensus estimate as of Alexion Investor Day on October 6, 2020

Sustainable Business Growing at >10% CAGR



Illustrative, non risk-adjusted revenues, peak sales year varies by program

[1]*2025 $9-10B target is at constant currencies (9/30/20 levels)*

Investor day

APPENDIX

Jesse living with gMG

Back-Up Slides

Late Stage Pipeline Timelines

Identifier	MOA	ROA	Indication	Phase	Study Start	Study End
SOLIRIS	Anti-C5	Q2W IV	Guillain Barre Syndrome	Ph3	Initiating 1H '21	Not yet disclosed
ULTOMIRIS (ravulizumab)	Anti-C5	Q1W SC	Paroxsymal Nocturnal Hemoglobinuria (PNH) Atypical Hemolytic Uremic Syndrome (aHUS)	Ph3	Initiated 1Q '19	TLR 2Q '20 Filing 3Q '21
		Q8W IV	Generalized Myasthenia Gravis (gMG)	Ph3	Initiated 1Q '19	TLR 2H '21
			Neuromyelitis Optica Spectrum Disorder (NMOSD)	Ph3	Initiated 4Q '19	TLR 1H '22
			Amyotrophic Lateral Sclerosis (ALS)	Ph3	Initiated 1Q '20	TLR 2H '22
			Hematopoetic Stem Cell Transplant Thrombotic Microangiopathy (HSCT-TMA)	Ph3	Initiating 4Q '20	Not yet disclosed
			Complement Mediated Thrombotic Microangiopathy (CM-TMA)	Ph3	Initiating 1H '21	Not yet disclosed
			Adults with COVID-19 who are hospitalized with severe pneumonia or ARDS	Ph3	Initiated 2Q '20	TLR 1H '21
			Renal Basket Study	Ph2	Initiating 4Q '20	Not yet disclosed
ALXN1720	Anti-C5 Bi-Specific	SC	Generalized Myasthenia Gravis (gMG)[1]	Ph1 HV	Reinitiated 3Q '20	TLR 1H '21
			Dermatomyositis (DM)[1]			
ALXN1840	Copper chelator	Oral	Wilson Disease	Ph3	Initiated 1Q '18	TLR 1H '21
ALXN1830	Anti-FcRn	SC	Warm Autoimmune Hemolytic Anemia (WAIHA)[2]	Ph1 HV	Reinitiating 1H '21	TLR 1H '21
			Generalized Myasthenia Gravis (gMG)[2]			
CAEL-101	ALκ/ALλ fibril reactive antibody	IV	Amyloid Light-Chain (AL) Amyloidosis	Ph3	Initiated 3Q '20	TLR 2H '22
AG10	TTR tetramers stabilizer (small molecule)	Oral	Transthyretin Amyloid Cardiomyopathy (ATTR-CM)	Ph3	Initiating 4Q '20	TLR 2H '22
ALXN2040	Factor D inhibitor (small molecule)	TID Oral	PNH with Extravascular Hemolysis (PNH w/ EVH)	Ph3	Initiating 4Q '20	TLR 2H '22
		TBD	Geographic Atrophy	Ph2	Initiating 2H '21	Not yet disclosed
ALXN2050	Factor D inhibitor (small molecule)	BID Oral	Paroxsymal Nocturnal Hemoglobinuria (PNH)	Ph2	Initiated 4Q '19	TLR 2H '21
			Renal Basket Study	Ph2	Initiating 1H '21	Not yet disclosed
ANDEXXA	Factor Xa Reversal	IV	Urgent Surgery	Ph2	Initiating 1H '21	Not yet disclosed
CERDULATINIB	SYK/JAK kinase inhibitor	Oral	Lymphoma (CTCL, PTCL, FL)	Ph2	PTLA Acquisition	TLR 1H '21

[1]1720 currently in HV Ph1 with topline readout estimated 1H '21 and subsequent DM and gMG trials to begin after that; [2]1830 Ph1 HV program to reinitiate for SC formulation with WAIHA and gMG Ph2 programs to follow in 2021

Near-Term Events Support All Three Pillars of Alexion's Value Creation Strategy

LEAD

US IPR Settlement (Soliris Patents)	2Q 2020	☑
ULTOMIRIS PNH Subcutaneous Ph3 Topline Data (PK)	2Q 2020	☑
ULTOMIRIS aHUS EMA Approval by EC	Mid 2020	☑
ULTOMIRIS 100mg/ml Formulation FDA Approval	2H 2020	☐
ULTOMIRIS Subcutaneous PNH/aHUS Launch	Mid 2022	☐

EXPAND

ULTOMIRIS HSCT-TMA Ph3 Trial Initiation	4Q 2020	☐
ULTOMIRIS Ph2 Renal Basket Trial Initiation	4Q 2020	☐
ULTOMIRIS COVID-19 Ph3 Data	1H 2021	☐
ULTOMIRIS gMG Ph3 Top Line Results	2H 2021	☐
ULTOMIRIS ALS Ph3 Top Line Results	1H 2022	☐
ULTOMIRIS NMOSD Ph3 Top Line Results	1H 2022	☐

DIVERSIFY

Portola Acquisition Close	3Q 2020	☑
ALXN2040 C3G Ph2 Data	Mid 2020	☑
AG10 Japan Ph3 Initiation	4Q 2020	☐
CAEL-101 Ph3 Trial Initiation	2H 2020	☑
ALXN1840 Wilson Ph3 Topline Data	1H 2021	☐
ALXN2050 PNH Ph2 Topline Data	2H 2021	☐
ALXN2040 GA Ph2 Initiation	2H 2021	☐
AG10 Japan Ph3 Top Line Results	2H 2022	☐
ALXN1840 Wilson Launch	2H 2022	☐

Growing and Advancing our Innovative Pipeline

2017	2018[3]	2019	2020
$736M Non-GAAP[1] **$878M** GAAP[1] R&D Expense	**$646M** Non-GAAP[1] **$730M** GAAP[1] R&D Expense	**$721M** Non-GAAP[1] **$886M** GAAP[1] R&D Expense	**$948M** Non-GAAP[1] **$1,040M** GAAP[1] R&D Expense[4]
2 Assets[2] *2 Internal*	**3 Assets** *1 Internal* *2 Through BD*	**8 Assets** *2 Internal* *6 Through BD*	**11 Assets** *3 Internal* *8 Through BD*
4 Clinical Programs	**8 Clinical Programs**	**14 Clinical Programs**	**20+ Clinical Programs**

Program Related Spend:

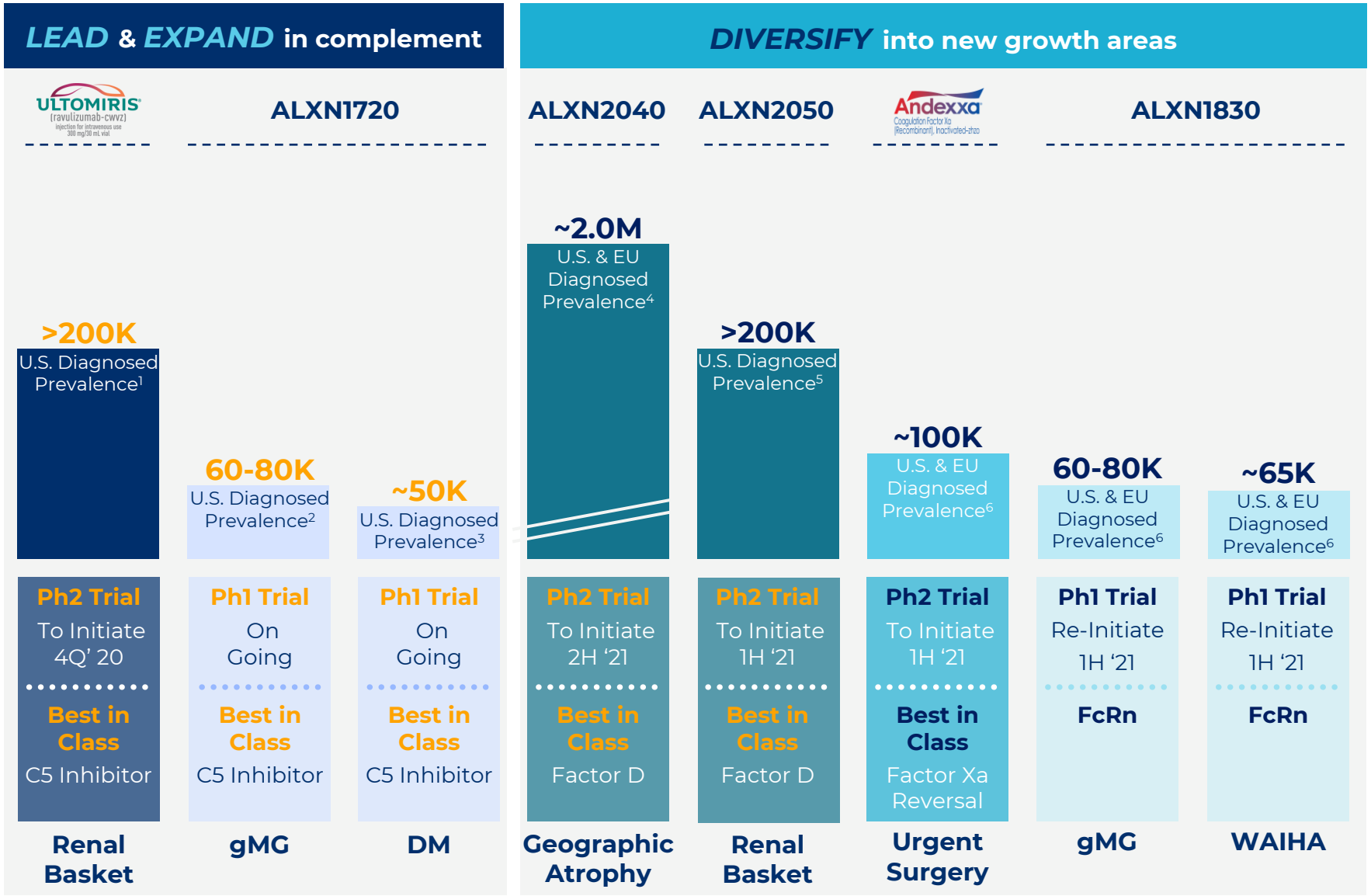
~50% C5 Expansion

~50% Non-C5 Diversification

Significant Progress Made Developing A Robust, Value-Creating Pipeline

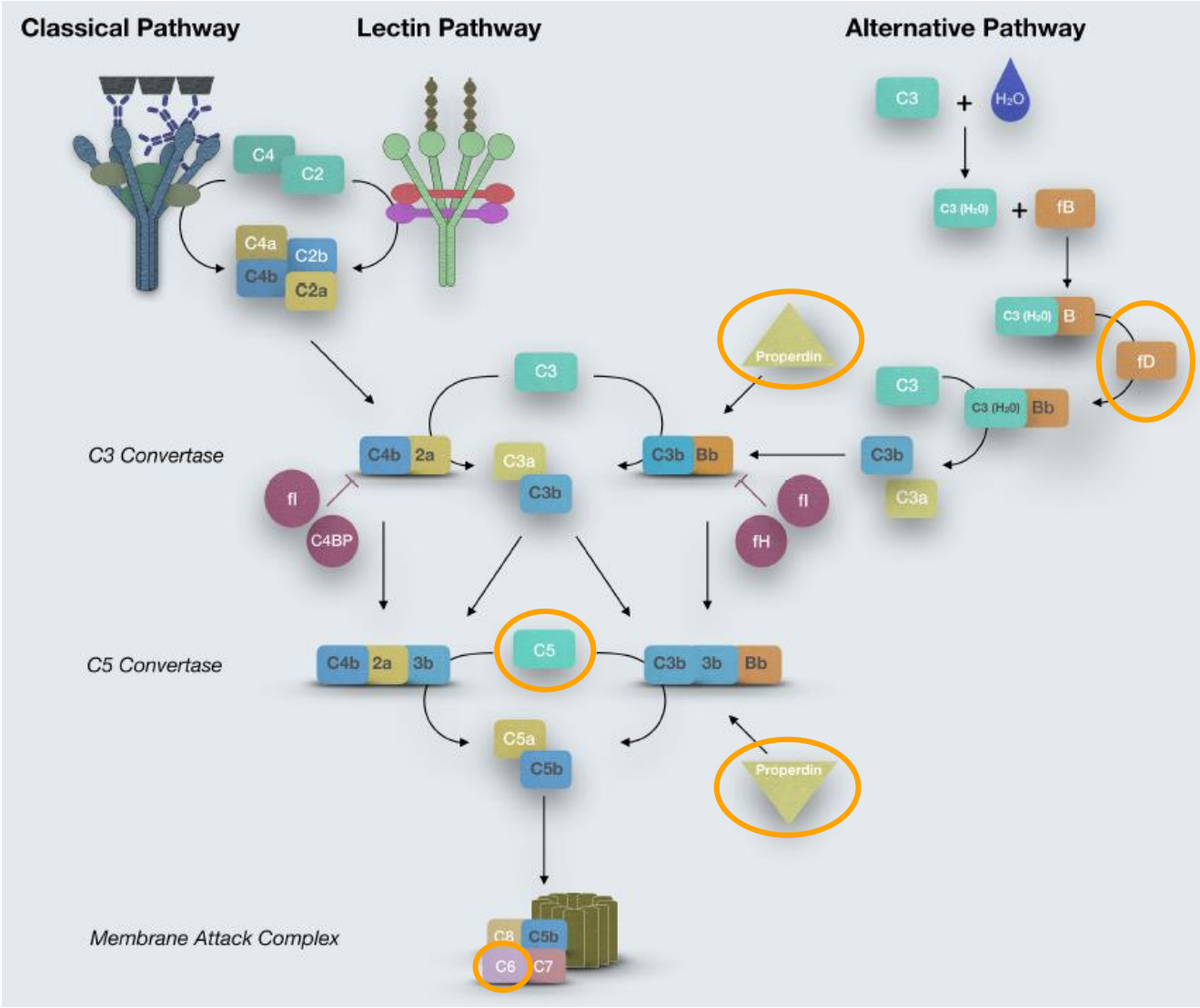
[1]A reconciliation of GAAP to non-GAAP financial results is provided in the appendix and is available at www.alexion.com.
[2]Asset defined as a singular molecular entity; [3]2018 decrease in R&D spend related to pipeline strategy refocus; [4]Mid point of 2020 guidance issued 7/30/20

Earlier Stage Pipeline Opportunities Beyond 2023 Launch



Targeting Diseases of Complement Dysregulation

- Complement is a master sensor that discriminates between foreign or altered and healthy cell surfaces
- Rationale for complement inhibition across multiple rare disease indications and therapeutic areas
- Complexity of complement biology allows for multiple targeting approaches
- Pursuing novel molecules and targets across terminal, lectin, and alternative pathways



Denotes target ALXN is investigating

Capital Allocation / Business Development

Updated Approach to Capital Allocation

Significant Progress Since 2017 Allows an Updated and More Flexible Approach

- Alexion constantly evaluates our capital allocation approach
- R&D portfolio has grown substantially since 2017
  - 20+ total programs
  - Confidence in potential of 10 launches by 2023 enabling capital return
- Free cash flow generation has improved over the last three years, with high FCF conversion[1]
- Allows Alexion to be more flexible on capital allocation strategy

Funding Expanding R&D Portfolio

↓

Capital Allocation Priorities

↓

Share Repurchase ⟷ **Balance Sheet (Reduce Net Debt)** ⟷ **Disciplined BD**

Committing to **$500 – $550M of repurchases** in 2020; increasing to **at least 1/3 of FCF** on average annually from 2021 - 2023

No near-term plan to pay a dividend given life-cycle

Near-term focus on successful Portola integration

Remain **open to future BD** that meet criteria for value creation and strategic fit

Robust Share Repurchase Program: Est. ~$3B & ~10% Reduction In Shares Outstanding Over Next Four Years[2]

(1) Free Cash Flow (FCF) defined as cash flow from operations less purchases of property, plant and equipment; FCF conversion defined as FCF divided by net income (2) Relative to 12/31/19 share count, excluding impact of new issuances

Diversifying our Portfolio through Clinical and Commercial BD

Asset	ANDEXXA *Factor Xa Reversal Agent*	ALXN1840 *(WTX-101) for treatment of Wilson Disease*	ALXN2040 & ALXN2050 *Two oral Factor D inhibitors*	ALXN1830 *(SYNT-001) an anti-FcRn molecule*	AG10 *Rights to develop for ATTR in Japan only*	CAEL-101 *Co-develop for AL Amyloidosis with option to acquire*	Elamipretide *Co-develop for mitochondrial diseases*	ABY-039 *Co-develop anti-FcRn molecule*
Date	2Q 2020	2Q 2018	4Q 2019	3Q 2018	3Q 2019	1Q 2019	4Q 2019	1Q 2019
Upfront $ *Net of Cash*	~$1,200M	~$810M	~$700M	~$400M	~$50M	~$30M	~$30M	~$25M
Named Indications	**Factor Xa Major Bleeds** ~700K bleeds per year	**Wilson** 10K US/EU Diagnosed	**PNH with EVH** <10% of PNH **Renal Basket** *Add'l indications in consideration*	**WAIHA** 65K US/EU Diagnosed **gMG** 60-80K US Diagnosed *Add'l indications in consideration*	**ATTR-CM** <6K Diagnosed Japanese Patients	**AL Amyloidosis** 10K US Diagnosed Patients		
Alexion Ambition	**Become standard of care** for all Factor Xa inhibitor patients with a major or life-threatening bleeds	**Redefine the standard of care** with a transformative, highly specific copper binding agent	Deliver **next generation "oral"** complement inhibitor in a range of larger, rare indications	To be a **leading SC FcRn** in a variety of auto-immune indications	Bring a **transformative small molecule** to treat the root cause of ATTR to patients in Japan	Be **the first targeted therapy** to address underlying disease for AL amyloidosis patients	Ph3 PMM program did not meet primary endpoint Did not exercise option to acquire	Ph1 Safety Signal Collaboration Terminated
Progress to Date	ANDEXXA on market in US and EU Wave 1 Expanding geographically and potential label expansions	Ph3 trial fully enrolled Re-powered for superiority and added key secondary endpoints	2040: Ph2 C3G data; PNH with EVH Ph3 to initiate 2H 20 2050: PNH Ph2 on-going; Ph 2 renal basket to initiate 1H 2021	WAIHA Ph2 IV initiated; will transition to SC Successful SC data showing IgG reduction through Ph1 SC HV to support future gMG study	Expanding Ph3 study into Japan in 2020	Ph2 on-going with successful dose finding Ph3 on track to initiate 2H 2020		

Above does not include deals made prior to 2018 (Taligen, Enobia, Xencor, Synageva, Halozyme) or pre-clinical deals/collaborations (Complement Pharma, Dicerna, Zealand, and Immune Pharma)

Pre-Clinical External Collaborations and Acquisitions Expand our Range of Expertise

Collaborations

Dicerna™

- Collaboration to discover and develop SC delivered GalXC™ RNAi therapies for complement-mediated diseases
  - Potential low volume injection with long half-life, supporting monthly+ dosing
- Initial agreement for two targets expanded to four in December 2019

ZEAL& ZEALAND PHARMA

- Partnership to co-discover a macrocyclic peptide inhibitor; platform already commercialized for other targets
- Peptide therapies offer a targeted approach with high potency and ease of administration
- Initial agreement for one target with option to expand to three additional

Complement pharma

- Partnership to co-discover a therapeutic antibody against C6
- Expands our complement franchise with potential to address neurological disorders
  - Circulating C6 in CNS leads to formation of MAC, which can cause neurodegeneration

Acquired

IMMUNE Pharmaceuticals

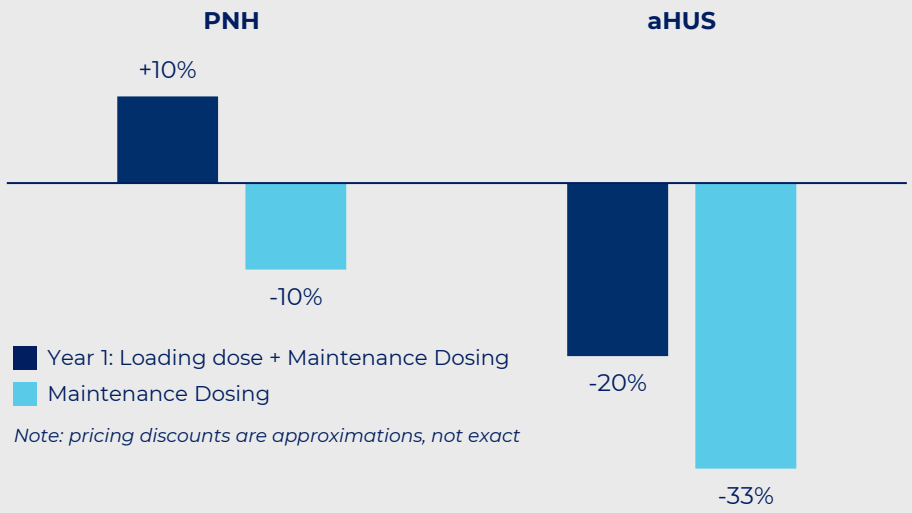
- Acquisition of ALXN2010 (anti-eotaxin-1) from Immune Pharma in 2019
- Eotaxin-1 is an eosinophil chemoattractant implicated in multiple inflammatory diseases
- Currently undergoing pre-clinical work to achieve subcutaneous administration

ULTOMIRIS Modeling / Pricing Considerations

ULTOMIRIS Conversion Dynamic: Two Key Considerations

Conversion Loading Dose Dynamic

ULTOMIRIS vs. SOLIRIS U.S. Annual Cost Per Patient



- SOLIRIS indication-specific dosing: aHUS, gMG, NMOSD labeled dose higher than PNH
  - *Drives indication-specific pricing differences when comparing SOLIRIS vs. ULTOMIRIS pricing*
- ULTOMIRIS weight-based dosing

Quarter-on-quarter (QoQ) Variability

Infusion Timing Drives QoQ Variability

Patient Sample 1: Loading dose + 2 Maintenance Infusions

Patient Sample 2: Loading dose + 1 Maintenance Infusion

Loading dose
Maintenance Infusion

- ULTOMIRIS every 8 week infusion schedule drives variability in quarterly patient treatment costs
- Expect quarterly variability to be negligible on year-over-year (YoY) revenue comparisons

The Alternative Complement Pathway & Renal Disease

The Alternative Pathway (AP) Is A Strong Mediator Of Abnormal Immune Response Across Several Renal Diseases

Sampling of Potential Basket Trial Indications

Lupus Nephritis (LN)
~90K US Prevalence

- LN is caused by pathologic complement-fixing immune complex deposits and the production of autoantibodies; **Complement triggers acute inflammation in kidney**
- Serum levels of complement biomarkers are linked with LN
- AP inhibition plays role in mediating these aberrant immune responses

IgA Nephropathy
~60K US Prevalence

- Alternative Pathway drives **inflammation and immune complex deposition** against under-glycosylated IgA in the kidney
- AP inhibition can prevent activation of complement response to immune complexes, with potential to treat underlying pathophysiology & improve clinical outcomes

PMN
~60K US Prevalence

- PMN is caused by pathologic auto antibodies against the PLA2R antigen on the podocyte surface of the kidney; over-amplifying the AP complement response
- Complement targeted therapy has potential to treat the underlying pathophysiology and improve clinical outcomes

C3G
<6K US Prevalence

- C3G is caused by **uncontrolled and continued activation alternative pathway**, causing C3 deposition and inflammation in the kidney leading to kidney damage
- Signals from ALXN2040 Ph2 C3G program support AP inhibition in C3G

Disease Descriptions

Alexion Current Indications

	Indication	Description	Links
PNH	Paroxysmal Nocturnal Hemoglobinuria	Chronic, debilitating, and potentially life-threatening ultra-rare blood disorder, with an average age of onset in the early 30s	more info
aHUS	atypical Hemolytic Uremic Syndrome	Ultra-rare, genetic, chronic, potentially life-threatening disease. Chronic uncontrolled complement activation results in thrombotic microangiopathy (TMA)	more info
gMG	Generalized Myasthenia Gravis	Debilitating, chronic, and progressive autoimmune neuromuscular disease.	more info
NMOSD	Neuromyelitis Optica Spectrum Disorder	Rare, devastating, complement-mediated disorder of the central nervous system characterized by relapses where each individual attack results in cumulative disability including blindness and paralysis, and sometimes premature death (primarily affects women)	more info
HPP	Hypophosphatemia	Inherited, progressive, ultra-rare metabolic disease in which patients experience devastating effects on multiple systems of the body, and face debilitating or life-threatening complications	more info
LAL-D	Liposomal Acid Lipase Deficiency	Genetic, chronic, and progressive ultra-rare metabolic disease in which infants, children, and adults experience continuous, uncontrolled accumulation of cholesteryl esters (CEs) and triglycerides (TGs) that may lead to multi-organ damage and premature death	more info
ANDEXXA	Coagulation factor Xa reversal (recombinant)	Reversal agent for life-threatening bleeds induced by factor Xa inhibitors	more info

Alexion Pipeline Indications - I

	Indication	Description	Links
WD	Wilson Disease	Rare, chronic, genetic, and potentially life-threatening liver disorder of impaired copper transport. The disorder is characterized by build-up of intra-cellular hepatic copper. Untreated, Wilson disease leads to various combinations and severity of hepatic, neurologic, and psychiatric symptoms, and can be fatal.	
ALA	AL (Light-chain) Amyloidosis	A protein misfolding disorder in which B-cells produce incomplete λ and κ light chain antibodies which clump in certain organs / tissues (including heart, lungs, kidneys, nervous system, and liver, eventually causing organ damage and death.	more info
PNH-EVH	Paroxysmal Nocturnal Hemoglobinuria with Extravascular Hemolysis	Chronic, debilitating, and potentially life-threatening ultra-rare blood disorder, with an average age of onset in the early 30s. EVH occurs when C3 opsonization of red blood cells causes macrophages to destroy those cells in tissue.	
DM	Dermatomyositis	Progressive autoimmune condition that causes skin changes and muscle weakness. Symptoms can include a red skin rash around the eyelids, red bumps around the joints, and muscle weakness in the arms and legs. Dermatomyositis is most common in adults between ages 40 and 60, or in children between ages 5 and 15.	more info
HSCT-TMA	Hematopoetic Stem Cell Transplant Thrombotic Micro-Angiopathy	A significant and often lethal complication of HSCT. The condition is a systemic, multifactorial disorder caused by endothelial cell damage induced by conditioning regimens, immunosuppressant therapies, infection, graft versus host disease (GVHD), and other factors associated with HSCT. HSCT-TMA prognosis is poor, with overall mortality reported as high as ~80-90%.	

Alexion Pipeline Indications - II

	Indication	Description	Links
CM-TMA	Complement-Mediated Thrombotic Micro-Angiopathy	Caused by abnormalities of regulation of the alternative pathway of complement activation. The indication describes a group of severe and chronic ultra-rare diseases that can cause progressive injury to vital organs— via damage to the walls of blood vessels and blood clots—potentially leading to organ failure and premature death. CM-TMA affects both adults and children and represents the population of patients with aHUS with or without triggers.	
COVID-19	Severe Acute Respiratory Distress Syndrome in COVID-19 patients	Patients with severe illness include those who are hospitalized with severe pneumonia or acute respiratory distress syndrome. Evidence suggests that acute lung injury associated with COVID-19 may be mediated in part by complement pathway whereby elevated C5 ultimately leads to severe pneumonia, blood clots and multi-organ dysfunction in many advanced COVID patients.	
WAIHA	Warm Auto-Immune Hemolytic Anemia	Rare autoimmune disorder caused by pathogenic Immunoglobulin G (IgG) antibodies that react with and cause the premature destruction of red blood cells at normal body temperature. The disease is often characterized by profound, and potentially life-threatening anemia and other acute complications.	
ATTR-CM	Transthyretin Amyloidosis (ATTR) with Cardiomyopathy (ATTR-CM)	A progressive, fatal disease caused by the accumulation of misfolded tetrameric transthyretin (TTR) amyloid in the heart. Caused by the destabilization of TTR due to inherited mutations or aging, symptoms usually manifest later in life (age 50+), with median survival of three to five years from diagnosis.	

Alexion Pipeline Indications - III

	Indication	Description	Links
LN	Lupus Nephritis	An inflammatory renal disease that is a severe complication of systemic lupus erythematosus (SLE), in which deposits of immune complexes (e.g., IgG and complement) accumulate in the kidney and lead to injury. Approximately 30% SLE patients develop LN, and up to 30% of patients are refractory to treatment and progress to end stage renal disease requiring dialysis/transplant within 15 years . There are no FDA approved therapies for LN.	
PMN	Primary Membranous Nephropathy	Rare autoimmune disease characterized by autoantibodies to the podocyte membrane antigens PLA2R (~85%) and THSD7A (~5%) that causes nephrotic syndrome and chronic kidney disease. Approximately 30% of patients will progress to end stage renal disease within 10 years of diagnosis.	
IgAN	IgA Nephropathy (IgAN)	A heterogenous disease in terms of clinical manifestations and progression and is the most common cause of primary glomerulonephritis. In IgAN, locally deposited immune complexes lead to activation of the complement cascade & downstream endothelial organ damage. The Lectin and Alternative Pathways are believed to be the main driver of disease progression, which includes end stage renal disease and need for dialysis or transplant.	
C3G	Complement 3 Glomerulopathy	Ultra-rare, heterogenous renal disease characterized by uncontrolled continued activation of fluid and/or solid phase alternative pathway causing C3 deposition and inflammation, leading to kidney damage .	
ALS	Amyotrophic lateral sclerosis	A rare neurological disorder of progressive deterioration of nerve cells (motor neurons) in the brain and the spinal cord that control muscles throughout the body. Loss of motor neurons and muscle strength leads to loss of independence, paralysis and death, typically due to respiratory insufficiency.	

Clinical Trials Appendix

ALXN-1840 (WTX-101)

Trial	**WTX101-301 trial** (NCT03403205)
Population:	Treatment-naïve and experienced Wilson disease patients
Arms:	Active: ALXN1840 15-60mg once-daily (1-4 15mg tablets 1x / day) Control: standard of care (trientine, penicillamine, Zinc or combinations)
Primary endpoint:	% change in non-ceruloplasmin-bound copper from baseline to week 48
Size (n) / randomization:	n=215, 1:1 randomization
Treatment period:	48 weeks + LTE, up to 60 mos total
Trial start date:	2/2018
Expected primary completion date:	2/2021
Clinicaltrials.gov link:	WTX101-301
Trial stage:	Phase 3

Directly sourced from www.clinicaltrials.gov

CAEL-101

Trial	CAEL101-301 trial (NCT04504825)	CAEL101-302 trial (NCT04512235)
Population:	1st line Mayo stage IIIb AL amyloidosis	1st line Mayo stage IIIa AL amyloidosis
Arms:	Active: CyBorD + CAEL-101 Control: CyBorD + Placebo	Active: CyBorD + CAEL-101 Control: CyBorD + Placebo
Primary endpoint:	Overall survival (OS)	Overall survival (OS)
Size (n) / randomization:	n=111, 2:1 randomization	n=267, 2:1 randomization
Treatment period:	Min 50 weeks	Min 50 weeks
Trial start date:	8/2020	8/2020
Expected primary completion date:	8/2022 (event-driven)	8/2022 (event-driven)
Clinicaltrials.gov link:	CAEL101-301	CAEL101-302
Trial stage:	Phase 3	Phase 3

Directly sourced from www.clinicaltrials.gov

ULTOMIRIS ALS & HSCT-TMA

Trial	ALXN1210-ALS-308 trial (NCT04248465)	ALXN1210-TMA-313 (NCT04543591)
Population:	Adult participants with diagnosed ALS (sporadic or familial); ALS onset ≤ 36 months from Screening	Adult and adolescent participants with hematopoietic stem cell transplant-associated thrombotic microangiopathy (HSCT-TMA)
Arms:	Active: ULTOMIRIS Control: Placebo	Arm 1: ULTOMIRIS + Best Supportive Care Arm 2: Best Supportive Care
Primary endpoint:	Change From Baseline In Amyotrophic Lateral Sclerosis Functional Rating Scale-Revised (ALSFRS-R) Total Score	TMA Response
Size (n) / randomization:	n=354, 2:1 randomization	n=184, 1:1 randomized open label
Treatment period:	50 weeks	26 weeks
Trial start date:	3/2020	9/2020
Expected primary completion date:	9/2022	8/2023
Clinicaltrials.gov link:	ALXN1210-ALS-308	ALXN1210-TMA-313
Trial stage:	Phase 3	Phase 3

Directly sourced from www.clinicaltrials.gov

ULTOMIRIS gMG & NMOSD

Trial	ALXN1210-MG-306 trial (NCT03920293)	ALXN1210-NMO-307 (NCT04170023)
Population:	Participants with generalized myasthenia gravis; MG-ADL ≥ 6 at screening and randomization	Adult participants with Anti-aquaporin-4 antibody-positive and a diagnosis of NMOSD; at least 1 attack or relapse in last 12 months
Arms:	Active: ULTOMIRIS Control: Placebo	Single Arm
Primary endpoint:	Change From Baseline In Myasthenia Gravis-Activities Of Daily Living (MG-ADL) Total Score	Time To First Adjudicated On-Trial Relapse
Size (n) / randomization:	n=160, 1:1 randomized	n=55
Treatment period:	26 weeks	26 weeks to 2 years
Trial start date:	3/2019	12/2019
Expected primary completion date:	12/2021	11/2021
Clinicaltrials.gov link:	ALXN1210-MG-306	ALXN1210-NMO-307
Trial stage:	Phase 3	Phase 3

Directly sourced from www.clinicaltrials.gov

ULTOMIRIS COVID-19

Trial	**ALXN1210-COV-305** (NCT04369469)
Population:	Adult patients with Coronavirus Disease 2019 (COVID-19) severe pneumonia, acute lung injury, or acute respiratory distress syndrome
Arms:	Active: ULTOMIRIS plus best supportive care Control: Best supportive care
Primary endpoint:	Survival
Size (n) / randomization:	n=270, 2:1 randomized open label
Treatment period:	29 days
Trial start date:	5/2020
Expected primary completion date:	1H 2021
Clinicaltrials.gov link:	ALXN1210-COV-305
Trial stage:	Phase 3

Directly sourced from www.clinicaltrials.gov

ALXN2040 (ACHN-4471) & ALXN2050 (ACHN5528)

Trial	**ALXN2040-PNH-301 trial** (NCT04469465)	**ACH228-110** (NCT04170023)
Population:	Add-on therapy in paroxysmal nocturnal hemoglobinuria with clinically evident extravascular hemolysis	Treatment-experienced and treatment–naïve PNH patients, as monotherapy
Arms:	Active: SOLIRIS or ULTOMIRIS + ALXN2040 (3x/day for ALXN2040) Control: SOLIRIS or ULTOMIRIS + Placebo (period I) and SOLIRIS or ULTOMIRIS + ALXN2040 (period II)	Single Arm
Primary endpoint:	Change in hemoglobin from baseline at week 12	Change in hemoglobin from baseline at week 12
Size (n) / randomization:	n=84, 2:1 randomization	n=26, open label
Treatment period:	12 weeks (+12 wks for period II) + 1 yr LTE	12 wks + LTE
Trial start date:	12/2020	12/2019
Expected primary completion date:	10/2022	7/2021
Clinicaltrials.gov link:	ALXN2040-PNH-301	ACH228-110
Trial stage:	Phase 3	Phase 2

Directly sourced from www.clinicaltrials.gov

Board of Directors & Senior Leadership

Board Leadership overview

Board Skillset

- 10 of 10 directors have public company experience
- **10 of 10 directors have experience acquiring and/or divesting businesses** and technologies and evaluating strategic corporate decisions
- **9 of 10 directors have C-suite level experience**, with **6 current or former CEOs**
- 8 of 10 directors have experience working for pharmaceutical or biopharmaceutical companies
  - One director is a highly respected and prominent biotechnology focused investor (Baker Bros.) and significant (2nd largest non-index) Alexion shareholder (4.05%)
  - One director is a former human resources executive who brings critical insight and experience to the design of executive compensation program

Board Committees

- Four standing Board committees - Audit and Finance, Leadership and Compensation, Nominating and Corporate Governance, and Science and Innovation
- S&I created to have full oversight over business development and internal / external R&D program goals and objectives by reviewing management's progress and performance in achieving goals and objectives and mitigating associated risks; all members highly experienced in biotech, medicine and investing

Best Practices

- Strong emphasis on Board independence and strong Board and committee involvement, provides robust independent oversight of management, enterprise risk (including ESG risks and opportunities), and corporate operations
- Separated the roles of Chairman of the Board and CEO

3 Biopharmaceutical Industry Veterans Added as New Directors Comprising of Former and Current CEOs

Appointed 4 New Independent Directors with Deep Industry Experience since September 2017 including three industry veterans who are former and current CEOs

Strengthened And Refreshed Our Board of Directors to Align with Corporate Strategy

Refreshed Board of Directors

Ludwig Hantson, Ph.D. ★
Chief Executive Officer
Tenure: 3 years
- Accomplished healthcare executive with more than 30 years of experience in the biopharmaceutical industry
- Director of Hologic
- Ph.D. in motor rehabilitation and physical therapy, master's degree in physical education, and a certification in high secondary education, all from the University of Louvain in Belgium

David Brennan
Independent Chairman of the Board
Tenure: 6 years
- Extensive experience as an executive leader in the pharmaceutical industry
- Significant industry and regulatory knowledge from a more than 39-year career in the pharmaceutical industry and serving as a director on multiple public company and industry trade group boards
- Former CEO of AstraZeneca; Director of Insmed
- B.A. in business administration from Gettysburg College

Andreas Rummelt, Ph.D.
Independent Director
Tenure: 10 years
- More than 20 years in executive management positions in the pharmaceutical industry
- Broad understanding of international business operations particularly with respect to manufacturing, quality and technical matters
- CEO of interPharmaLink
- Ph. D in pharmaceutical sciences from the University of Erlangen-Nurernberg (Germany)

Chris Coughlin
Independent Director
Tenure: 6 years
- Extensive experience in complex financial and accounting matters, including public accounting and reporting
- Extensive operational experience managing, as well as evaluating and developing strategic goals for complex global organizations
- Former EVP and CFO of Tyco; extensive public company board experience; named NACD Director of the Year in 2015.
- B.S. in accounting from Boston College

Jack Mollen
Independent Director
Tenure: 6 years
- Significant experience in executive compensation policy and administration including more than 30 years as chief human resources senior executive
- Valued perspectives to the Board on matters of talent, executive compensation, benefits and leadership
- Former EVP for Human Resources of EMC Corp.
- B.A. in Economics from St. John Fisher College and a Master's degree in Labor Relations from St. Francis College

Felix Baker, Ph.D.
Independent Director
Tenure: 5 years
- Broad experience serving as both a director and investor of biotechnology companies providing a strategic perspective of the industry
- Significant industry and product development knowledge from a more than 25-year career investing in biotechnology
- Co-Managing Member of Baker Bros. Advisors; Lead Independent Director of Kodiak Sciences and Kiniksa Pharmaceuticals; Director of Seattle Genetics
- Ph. D and B.S. in Immunology from Stanford University

Dr. Francois Nader, M.D. ★
Independent Director
Tenure: 3 years
- Experienced biopharmaceutical CEO with strong background across both commercial and R&D functions
- Deep experience investing in biotechnology companies providing valuable insight in evaluating internal development product initiatives and external opportunities
- Former CEO of NPS Pharma; Chairman of Prevail Therapeutics and Acceleron Pharma; Director of Moderna
- Doctorate in Medicine from St. Joseph University (Lebanon) and MBA from the University of Tennessee

Dr. Paul Friedman, M.D. ★
Independent Director
Tenure: 3 years
- Deep experience in research and clinical development
- Extensive experience building and leading R&D organizations, expanding company pipelines of assets, and overseeing the commercial development of innovative therapeutic products
- Chairman and CEO of Madrigal Pharmaceuticals and Director of Incyte
- A.B. in Biology from Princeton University and M.D. from Harvard Medical School

Judy Reinsdorf ★
Independent Director
Tenure: 2 years
- Strong corporate governance, compliance and legal expertise from leading legal functions at large U.S. public companies with global operations and in regulated industries
- Broad experience in global compliance, strategic planning, data privacy, regulatory matters, as well as global M&A experience
- Former EVP and General Counsel of Johnson Controls
- Bachelor's degree from the University of Rochester and J.D. from Cornell Law School

Dr. Deborah Dunsire, M.D. ★
Independent Director
Tenure: 3 years
- More than 30 years of experience in the biopharmaceutical industry, including experience as a CEO if innovative companies focused on drug research and development
- Extensive experience leading complex drug discovery, development and commercialization organizations
- President and CEO of Lundbeck; Director of Ultragenyx
- Medical degree from the University of Witwatersrand, Johannesburg, South Africa

5 of the 9 independent directors have been appointed in the last three years

★ **Director added since March 2017**

New Management Team Assembled Since 2017

Ludwig Hantson, Ph.D.
Chief Executive Officer
- Accomplished healthcare executive with more than 30 years of experience in the biopharmaceutical industry
- Ph.D. in motor rehabilitation and physical therapy, master's degree in physical education, and a certification in high secondary education, all from the University of Louvain in Belgium

Joined EC: March 2017

Aradhana Sarin, M.D.
Executive Vice President, Chief Financial Officer
- Responsible for overseeing global financial management, treasury, internal audit, corporate strategy, business development, investor relations, security activities, and business operations, including corporate planning
- Completed medical training at the University of Delhi and received her MBA from Stanford Business School

Joined EC: February 2019

John Orloff, M.D.
Executive Vice President, Head of Research & Development
- Responsible for global R&D, Regulatory, and Medical Affairs at Alexion; including enhancing R&D productivity and supporting business development
- Bachelor of Arts from Dartmouth College, and a M.D. from the University of Vermont College of Medicine. Completed medical training at the University of Pittsburgh Medical Center and Yale University School of Medicine

Joined EC: June 2017

Brian Goff
Executive Vice President, Chief Commercial & Global Operations Officer
- Leads the global commercial and operations teams, which includes responsibility for country operations in each of Alexion's affiliates in North America, Europe, Japan, Asia Pacific, and Latin America
- MBA from the Wharton School at the University of Pennsylvania and a Bachelor of Arts from Skidmore College

Joined EC: June 2017

Tanisha Carino, Ph.D.
Executive Vice President, Chief Corporate Affairs Officer
- Responsible for global government relations, policy and communications
- Ph.D. in health policy from Johns Hopkins University, and is associate faculty at the Johns Hopkins Bloomberg School of Public Health

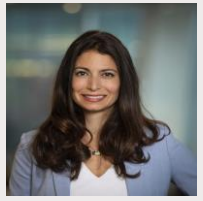
Joined EC: November 2019

Ellen Chiniara, J.D.
Executive Vice President, Chief Legal Officer and Corporate Secretary
- Responsible for overseeing all global legal matters for the Company
- J.D. from Stanford University's School of Law and Bachelor's Degree from Bryn Mawr College, as well as a graduate fellow at Yale University in Slavic Languages

Joined EC: February 2018

Indrani Franchini, J.D.
Executive Vice President, Chief Compliance Officer
- Responsible for leading Alexion's global compliance program and co-leads the Global Corporate Compliance Committee
- J.D. from the University of Michigan Law School and a Bachelor of Arts from Princeton University, as well as a Fulbright Fellow at Kyushu University

Joined EC: June 2017



Rana Strellis
Senior Vice President, Global Culture and Corporate Social Responsibility
- Rana Strellis is Senior Vice President, Global Culture and Corporate Social Responsibility of Alexion.
- MBA from Cornell University and BA in Economics from the University of Illinois at Urbana-Champaign

Joined EC: October 2017

Morgan Sanford
Chief of Staff to the Chief Executive Officer
- Organizes and prioritizes critical issues and required information for the CEO and Executive Committee
- MBA from the Leonard N. Stern School at New York University and her Bachelor's degree in Neuroscience from Hamilton College

Joined EC: March 2020

Uzair Qadeer
Chief Diversity Officer
- Responsible for cultivating an environment that fosters Diversity, Inclusion, and Belonging
- M.S. B.S. and B.A. from Pennsylvania State University

Joined EC: August 2020

Becky Lillie
Interim Chief Human Experience Officer
- Bec Lillie is the interim Chief Human Experience Officer at Alexion. In this role, she is responsible for the technology experience, patient experience and employee experience to accelerate Alexion's vision to become the No.1 patient centric rare disease company.
- BA in Communications from University of North Dakota

Joined EC: August 2020

7 of 11 Executive team members are women; 53% of total workforce are female

Alexion is one of three S&P 500 companies where women are a majority of executives

Sustainability At Alexion

Alexion's COVID-19 Response

Our Ongoing Commitment

To the Broader Community:

- Alexion is committed to assisting communities and medical systems in their efforts to address the public health crisis caused by the COVID-19 pandemic and is taking a holistic approach to supporting our global communities across a range of areas of need.

To Our Patients:

- We remain committed to our mission of serving patients with rare disease
- We remain focused on continuous supply of our medicines to our patients who rely on them
- We are steadfast in ensuring the integrity of our ongoing clinical trials and mitigating risks of interruption

To Our Employees:

- We continue to be dedicated to the health and safety of our global employees

Alexion's Response

Initiated Phase 3 Randomized Controlled Trial:

- Phase 3 trial investigating the use of ULTOMIRIS in patients with severe COVID-19
- Early pre-clinical and anecdotal evidence from independent investigator-sponsored work suggests a role for complement in treating severe COVID-19

Providing Support:

- Opened emergency Expanded Access programs for some patients' urgent needs
- Leveraged robust supply chain to ensure sufficient inventory for critical life-saving medications
- Remain committed to support community and local healthcare efforts to address COVID-19 public health crisis, including donation of lab equipment and instruments to hospital labs
- Alexion Charitable Foundation donated $500,000 across three nonprofit organizations to global COVID-19 response funds
- All non-essential employees (not in labs or manufacturing) have worked from home to ensure their safety; established policies / procedures to support more workday flexibility
- Programs put in place to foster employees' connectedness and provide support needed.

Corporate Social Responsibility (CSR) at Alexion

ALEXION® CSR-S·T·A·R

SUPPORTING OUR MISSION TO TRANSFORM THE LIVES OF PEOPLE AFFECTED BY RARE AND DEVASTATING DISEASE WHILE CREATING VALUE FOR ALL OUR STAKEHOLDERS.

SERVE
COMMUNITIES AND SUSTAIN OUR PLANET

We invest in our communities and shared planet in support of those who depend on us today and for generations that follow.

TRANSFORM
PATIENT LIVES

We urgently seek to understand patient journeys, find answers, and collaborate to deliver access to therapies that change lives.

ADVANCE
OUR PEOPLE AND OUR COMPANY

We aspire to become the most rewarding company to work for, embracing belonging, and governing and managing our business to return value to our stakeholders.

REDEFINE
WHAT IT MEANS TO LIVE WITH A RARE DISEASE

We pioneered complement biology, spurring new treatments for devastating disorders. We work to advance healthcare through innovative diagnostics and proactive transparency.

ETHICS & COMPLIANCE: OUR FOUNDATION

We build trust when we make the right choices and act with integrity. Our unwavering commitment to ethics, quality and compliance improves our ability to serve patients and enhances our reputation and competitive advantage.

CSR IS AN ACRONYM FOR CORPORATE SOCIAL RESPONSIBILITY

Recognition

Corporate ESG Performance Prime
RATED BY ISS ESG

Awarded to companies with an ESG performance above the sector-specific Prime threshold, which means that they fulfil ambitious absolute performance requirements.

SUSTAINALYTICS

	Rank (1st = lowest risk)	Percentile (1st = lowest risk)
Global Universe	4300 out of 12781	34th
Pharmaceuticals (Industry Group)	47 out of 751	7th
Biotechnology (Subindustry)	5 out of 332	2nd

Corporate Social Responsibility (CSR) at Alexion

In 2019, Alexion formalized aspirations and accompanying metrics aligned with our CSR-STAR Platform. Even though these formal aspirations and metrics were recently established, our teams have been committed to many of these areas of focus since the company's founding. For most areas, 2019 will serve as the baseline year against which we will measure progress, with the exception of two environmental metrics for which the baseline year will be 2020. Moving forward, we plan to report yearly progress against these aspirations in our annual CSR report.

FOCUS AREA	ASPIRATION	METRIC	2019 STATUS
SERVE COMMUNITIES AND SUSTAIN OUR PLANET	Make a positive impact in our communities	Increase year-over-year engagement in Global Day of Service, our primary employee volunteer event	13% increase in employee participation over 2018
	Be responsible stewards of our shared environment	Reduce greenhouse gas emissions (absolute and intensity)	Establishing baseline in 2020
		Increase amount of water recycled and reused	Establishing baseline in 2020
		Maintain zero process waste-to-landfill enterprise-wide year over year	Ongoing
TRANSFORM PATIENT LIVES	Support people affected by rare and devastating diseases and their caregivers throughout the patient journey	Increase the percentage of eligible patients who have been diagnosed with a disease that Alexion treats, that are included in Alexion patient support programs year over year, regardless of whether they are using our therapies	Nearly 70% of new, international patient support programs established in 2019 included patients not currently on Alexion therapies
		Ensure that, where possible, ongoing and new patient support programs have patient organizations involved in development, design and implementation	All international patient support programs initiated in 2019 included patient organization input
		Generate real-world evidence to document the benefits and safety of Alexion treatments	Ongoing

FOCUS AREA	ASPIRATION	METRIC	2019 STATUS
TRANSFORM PATIENT LIVES	Support people affected by rare and devastating diseases and their caregivers throughout the patient journey	Include patient and caregiver perspective, advice and input into early clinical development plans and identified Phase II/III Alexion clinical trials to inform protocols and where possible, patients' informed consent documents	Ongoing
		Be a key influencer of patient organization engagement standards across the industry through ongoing, proactive thought leadership	Ongoing
ADVANCE OUR PEOPLE AND OUR COMPANY	Become the most rewarding company to work for	Implement key culture initiatives to drive patient-centricity year over year	Ongoing
		Advance employee experience priorities to drive patient-centricity year over year	Ongoing
		Create more patient-centric decision-making	Ongoing
REDEFINE WHAT IT MEANS TO LIVE WITH A RARE DISEASE	Advance healthcare through new therapies and innovative diagnostics	Accelerate diagnostics for rare disease patients by creating or optimizing new tests for the patient community, year over year	Ongoing
		Pursue programs to bring novel therapies to patients, year over year	Ongoing
ETHICS & COMPLIANCE: OUR FOUNDATION	Build trust through ethics, quality and compliance	Be a key influencer of compliance standards across the industry through ongoing, proactive thought leadership	Ongoing

CSR Reporting Principles

- Utilize **best practice frameworks** to anchor content selection.
- Focus on **Shared Value** – the premise that companies can profit by doing "good," supported by examples of **Alexion's core business** activities.
- Proactively share information sought by **internal and external stakeholders**.
- Develop a tool that may be used by our colleagues to **tell our Alexion CSR story**.
- Create a narrative that instills employees with **pride** and external stakeholders with **trust**.

Sustainability Accounting Standards Board (SASB)

- Gaining traction, considered advanced
- Tailored to investors and lenders
- Highly sector specific

Global Reporting Initiative (GRI) Standards

- Most widely adopted CSR reporting framework in the world
- Used by 75% of the world's largest companies
- Caters to information needs of a broad cross section of stakeholders

Our CSR Evolution

Q3 2017 – 2019

- Created and launched **Alexion's CSR-STAR Platform** and long-term, sustainable strategy
- **Translated CSR-STAR** into 8 languages
- Created **CSR-Steering Committee** & Grew **CSR Team**
- Participated in internal & external **CSR education and awareness** events
- Engaged for first time with external **CSR Rating/Rankings** agencies
- Established membership with key external **CSR industry groups**

First Half | 2020

- Launched **Volunteer Tracking Platform** & **Volunteer Paid Time Off**
- Launched **Alexion Charitable Foundation (ACF)**
- Deployed **COVID-19 philanthropic relief** (Corporate Giving & ACF)
- Launched first **CSR Report**
- Launched **COVID-19 virtual volunteering program**
- Kicked-off Alexion's **Global Brain Health strategy** development
- Kicked-off creation of global **CSR overview video** for use with internal and external stakeholders

Second Half | 2020

- Completing 2020 CSR **Benchmarking Assessment**
- Identifying **CSR/ESG Reporting Gaps** and Alexion-specific **areas of focus**
- Leading Alexion's first **Virtual Global Week of Service**
- Developing **ACF Thought Leadership Platform** around mental health within the Rare Disease Community
- Develop ACF Global **Disaster Relief Policy** (Planned)
- Expand Alexion's **Matching Gifts** program (Planned)

Diversity, Inclusion & Belonging

At Alexion, Diversity is having a seat at the table. Inclusion is having a voice. Belonging is having that voice be heard. Ensuring all of our colleagues feel a sense of belonging will enable us to magnetize and incubate diverse talent which will allow us to harness diverse insights that fuel innovation and create value for the patients we serve.

Uzair Qadeer
Chief Diversity Officer

Uzair Qadeer is Chief Diversity Officer of Alexion. In this role, he is responsible for cultivating an environment that fosters Diversity, Inclusion, and Belonging.

"Establishing the Chief Diversity Officer role at Alexion is an important next step in our continued efforts to cultivate diversity, inclusion and a unique sense of belonging at the company, all of which enhances our ability to deliver on our mission of transforming the lives of patients with rare diseases and devastating conditions."

-Ludwig Hantson, Ph.D., CEO, Alexion

53%
of Alexion's total workforce are female

MassBio
MASSACHUSETTS BIOTECHNOLOGY COUNCIL

Our Commitment: The MassBio CEO Pledge for a More Equitable and Inclusive Life Sciences Industry

2Q 2020
GAAP to Non-GAAP Reconciliation

ALEXION PHARMACEUTICALS, INC.
TABLE 1: CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(in millions, except per share amounts)
(unaudited)

	Three months ended June 30,		Six months ended June 30,	
	2020	2019	2020	2019
Net product sales	$ 1,444.5	$ 1,202.5	$ 2,889.1	$ 2,342.7
Other revenue	0.1	0.8	0.3	1.0
Total revenues	1,444.6	1,203.3	2,889.4	2,343.7
Costs and expenses:				
Cost of sales (exclusive of amortization of purchased intangible assets)	144.9	99.2	256.6	185.0
Research and development	221.1	187.6	422.0	383.5
Selling, general and administrative	301.4	299.3	621.3	580.8
Acquired in-process research and development	—	(4.1)	—	(4.1)
Amortization of purchased intangible assets	73.7	80.1	147.4	160.1
Change in fair value of contingent consideration	15.8	6.1	21.6	(22.6)
Acquisition-related costs	4.6	—	42.7	—
Restructuring expenses	—	2.5	(0.8)	11.6
Impairment of intangible assets	2,053.3	—	2,053.3	—
Total costs and expenses	2,814.8	670.7	3,564.1	1,294.3
Operating (loss) income	(1,370.2)	532.6	(674.7)	1,049.4
Other income and expense:				
Investment income (expense)	41.5	(14.9)	36.3	27.6
Interest expense	(23.6)	(18.3)	(49.4)	(38.2)
Other income and (expense)	0.2	0.1	(0.7)	2.5
(Loss) income before income taxes	(1,352.1)	499.5	(688.5)	1,041.3
Income tax (benefit) expense	(284.0)	39.7	(178.0)	(6.4)
Net (loss) income	$(1,068.1)	$ 459.8	$ (510.5)	$ 1,047.7
Earnings (loss) per common share				
Basic	$ (4.84)	$ 2.05	$ (2.31)	$ 4.68
Diluted	$ (4.84)	$ 2.04	$ (2.31)	$ 4.64
Shares used in computing earnings (loss) per common share				
Basic	220.6	224.2	221.1	224.0
Diluted	220.6	225.6	221.1	225.7

ALEXION PHARMACEUTICALS, INC.
TABLE 2: RECONCILIATION OF GAAP TO NON-GAAP FINANCIAL RESULTS
(in millions, except per share amounts)
(unaudited)

	Three months ended June 30,		Six months ended June 30,	
	2020	2019	2020	2019
GAAP net (loss) income	$(1,068.1)	$ 459.8	$ (510.5)	$ 1,047.7
Before tax adjustments:				
Cost of sales:				
Share-based compensation	3.1	3.5	6.2	7.3
Research and development expense:				
Share-based compensation	16.5	13.9	31.7	29.2
Upfront payments related to licenses and other strategic agreements [1]	—	25.0	—	46.2
Selling, general and administrative expense:				
Share-based compensation	47.8	43.5	87.1	81.2
Litigation charges [2]	—	—	21.5	0.1
Acquired in-process research and development	—	(4.1)	—	(4.1)
Amortization of purchased intangible assets	73.7	80.1	147.4	160.1
Change in fair value of contingent consideration [3]	15.8	6.1	21.6	(22.6)
Acquisition-related costs [4]	4.6	—	42.7	—
Restructuring expenses	—	2.5	(0.8)	11.6
Impairment of intangible assets[5]	2,053.3	—	2,053.3	—
Investment income (expense):				
(Gains) and losses related to strategic equity investments	(35.0)	25.2	(25.8)	(8.6)
Other income and (expense):				
Adjustments to income tax expense [6]	(409.5)	(50.5)	(444.7)	(197.5)
Non-GAAP net income	$ 702.2	$ 605.0	$ 1,429.7	$ 1,150.6
GAAP earnings (loss) per common share - diluted	$ (4.84)	$ 2.04	$ (2.31)	$ 4.64
Non-GAAP earnings per common share - diluted	$ 3.11	$ 2.64	$ 6.33	$ 5.04
Shares used in computing diluted earnings (loss) per common share (GAAP)	220.6	225.6	221.1	225.7
Shares used in computing diluted earnings per common share (non-GAAP)	225.7	228.9	225.9	228.5

(1) During the three months ended June 30, 2019, we recorded expense of $25.0 million in connection with an upfront payment on a strategic agreement that we entered into with Affibody AB (Affibody). During the six months ended June 30, 2019, we recorded expense of $46.2 million in connection with upfront payments on strategic agreements that we entered into with Affibody and Zealand Pharma A/S.

(2) During the six months ended June 30, 2020, we recorded $21.5 million in litigation charges in connection with legal proceedings.

(3) Changes in the fair value of contingent consideration expense for the three and six months ended June 30, 2020 as well as the six months ended June 30, 2019 include the impact of changes in the expected timing of achieving contingent milestones, in addition to the interest component related to the passage of time. For the three months ended June 30, 2019, changes in fair value of contingent consideration expense reflected only the interest component of contingent consideration related to the passage of time.

(4) For the three and six months ended June 30, 2020, we recorded $4.6 million and $42.7 million, respectively, of acquisition-related costs in connection with the Achillion Pharmaceuticals, Inc. and Portola Pharmaceuticals, Inc. acquisitions. Acquisition-related costs primarily consist of Achillion and Portola transaction costs, costs associated with the accelerated vesting of stock options previously granted to Achillion employees and Achillion restructuring-related costs.

(5) In the second quarter 2020, we recognized impairment charges of $2,053.3 million, primarily related to our KANUMA intangible asset.

(6) Alexion's non-GAAP income tax expense for the three and six months ended June 30, 2020 and 2019 excludes the tax effect of pre-tax adjustments to GAAP profit. Non-GAAP income tax expense for the six months ended June 30, 2019 also excludes certain one-time tax benefits of $95.7 million and $30.3 million associated with a tax election made with respect to intellectual property of Wilson and a release of an existing valuation allowance, respectively.

ALEXION PHARMACEUTICALS, INC.
TABLE 3: RECONCILIATION OF GAAP TO NON-GAAP FINANCIAL GUIDANCE
(in millions, except per share amounts and percentages)
(unaudited)

	Twelve months ending December 31, 2020	
	Low	High
GAAP net income	$ 214	$ 290
Before tax adjustments:		
Share-based compensation	295	282
Impairment of intangible assets	2,053	2,053
Amortization of purchased intangible assets	202	202
Acquisition-related costs	131	131
Change in fair value of contingent consideration	31	31
Restructuring expenses	(1)	(1)
(Gains) and losses related to strategic equity investments	(26)	(26)
Litigation charges	22	22
Adjustments to income tax expense	(519)	(515)
Non-GAAP net income	$ 2,402	$ 2,469
Diluted GAAP earnings per common share	$ 0.96	$ 1.30
Diluted non-GAAP earnings per common share	$ 10.65	$ 10.95
Costs and expenses and margin (% total revenues)		
GAAP research and development expense	19.2 %	18.1 %
Share-based compensation	1.7 %	1.6 %
Restructuring related expenses	0.0 %	0.0 %
Non-GAAP research and development expense	17.5 %	16.5 %
GAAP selling, general and administrative expense	25.7 %	24.5 %
Share-based compensation	3.3 %	3.1 %
Restructuring related expenses	0.0 %	0.0 %
Litigation charges	0.4 %	0.4 %
Non-GAAP selling, general and administrative expense	22.0 %	21.0 %
GAAP operating margin	3.8 %	5.4 %
Share-based compensation	5.3 %	5.0 %
Litigation charges	0.4 %	0.4 %
Impairment of intangible assets	37.0 %	36.7 %
Amortization of purchased intangible assets	3.6 %	3.6 %
Acquisition-related costs	2.4 %	2.3 %
Change in fair value of contingent consideration	0.6 %	0.6 %
Restructuring expenses	0.0 %	0.0 %
Non-GAAP operating margin	53.0 %	54.0 %
Income tax expense (% of income before income taxes)		
GAAP income tax expense (benefit)	(26.0)%	(27.0)%
Tax effect of pre-tax adjustments to GAAP net income	42.5 %	42.5 %
Non-GAAP income tax expense	16.5 %	15.5 %

Amounts may not foot due to rounding.

ALEXION PHARMACEUTICALS, INC.
TABLE 4: NET PRODUCT SALES BY GEOGRAPHY
(in millions)
(unaudited)

	Three months ended June 30,		Six months ended June 30,	
	2020	2019	2020	2019
SOLIRIS				
United States	$ 553.3	$ 496.3	$ 1,109.5	$ 960.0
Europe	247.9	280.2	511.4	544.7
Asia Pacific	82.4	110.3	169.5	211.2
Rest of World	91.9	94.0	208.0	226.9
Total SOLIRIS	$ 975.5	$ 980.8	$ 1,998.4	$ 1,942.8
ULTOMIRIS				
United States	$ 158.1	$ 54.2	$ 289.6	$ 78.8
Europe	32.0	—	65.8	—
Asia Pacific	59.6	—	116.7	—
Rest of World	1.4	—	1.8	—
Total ULTOMIRIS	$ 251.1	$ 54.2	$ 473.9	$ 78.8
STRENSIQ				
United States	$ 140.7	$ 106.2	$ 268.8	$ 205.7
Europe	18.3	19.5	42.3	37.0
Asia Pacific	15.0	12.1	28.6	22.0
Rest of World	10.3	3.5	16.8	6.7
Total STRENSIQ	$ 184.3	$ 141.3	$ 356.5	$ 271.4
KANUMA				
United States	$ 15.4	$ 15.3	$ 31.8	$ 29.1
Europe	8.4	6.8	15.9	13.1
Asia Pacific	0.9	1.3	1.8	2.1
Rest of World	8.9	2.8	10.8	5.4
Total KANUMA	$ 33.6	$ 26.2	$ 60.3	$ 49.7
Net Product Sales				
United States	$ 867.5	$ 672.0	$ 1,699.7	$ 1,273.6
Europe	306.6	306.5	635.4	594.8
Asia Pacific	157.9	123.7	316.6	235.3
Rest of World	112.5	100.3	237.4	239.0
Total Net Product Sales	$ 1,444.5	$ 1,202.5	$ 2,889.1	$ 2,342.7

ALEXION PHARMACEUTICALS, INC.
TABLE 5: CONDENSED CONSOLIDATED BALANCE SHEETS
(in millions)
(unaudited)

	June 30, 2020	December 31, 2019
Cash and cash equivalents	$ 2,825.0	$ 2,685.5
Marketable securities	26.8	64.0
Trade accounts receivable, net	1,372.2	1,243.2
Inventories	577.7	627.6
Prepaid expenses and other current assets	566.2	456.1
Property, plant and equipment, net	1,196.4	1,163.3
Intangible assets, net	2,059.7	3,344.3
Goodwill	5,075.2	5,037.4
Right of use operating assets	209.9	204.0
Deferred tax assets	2,332.4	2,290.2
Other assets	461.7	429.0
Total assets	$ 16,703.2	$ 17,544.6
Accounts payable and accrued expenses	$ 861.6	$ 966.7
Current portion of long-term debt	126.8	126.7
Other current liabilities	131.7	100.9
Long-term debt, less current portion	2,311.6	2,375.0
Contingent consideration	374.7	192.4
Deferred tax liabilities	1,946.8	2,081.4
Noncurrent operating lease liabilities	169.4	164.1
Other liabilities	289.8	265.6
Total liabilities	6,212.4	6,272.8
Total stockholders' equity	10,490.8	11,271.8
Total liabilities and stockholders' equity	$ 16,703.2	$ 17,544.6

ALEXION PHARMACEUTICALS, INC.
TABLE 6: CONDENSED CONSOLIDATED STATEMENT OF CASH FLOWS
(in millions)(unaudited)

	Six months ended June 30,	
	2020	2019
Cash flows from operating activities:		
Net (loss) income	$ (510.5)	$ 1,047.7
Adjustments to reconcile net (loss) income to net cash flows from operating activities:		
Depreciation and amortization	179.1	193.7
Change in fair value of contingent consideration	21.6	(22.6)
Share-based compensation expense	125.0	117.6
Deferred taxes (benefit)	(226.6)	(40.8)
Unrealized foreign currency loss (gain)	3.3	(4.1)
Unrealized (gain) loss on forward contracts	(11.5)	11.3
Unrealized gain on strategic equity investments	(25.8)	(8.6)
Inventory obsolescence charge	17.2	—
Impairment of intangible assets	2,053.3	—
Other	10.5	(2.3)
Changes in operating assets and liabilities, excluding the effect of acquisitions:		
Accounts receivable	(137.6)	(196.4)
Inventories	(15.1)	(24.0)
Prepaid expenses, right of use operating assets and other assets	(54.8)	(126.8)
Accounts payable, accrued expenses, lease liabilities and other liabilities	(88.5)	23.6
Net cash provided by operating activities	1,339.6	968.3
Cash flows from investing activities:		
Purchases of available-for-sale debt securities	(19.4)	(41.1)
Proceeds from maturity or sale of available-for-sale debt securities	166.3	139.3
Purchases of mutual funds related to nonqualified deferred compensation plan	(9.5)	(10.9)
Proceeds from sale of mutual funds related to nonqualified deferred compensation plan	5.3	9.0
Purchases of property, plant and equipment	(18.4)	(82.8)
Payment for acquisition of business, net of cash acquired	(837.7)	—
Purchases of strategic equity investments and options	(38.1)	(43.8)
Purchase of intangible assets	—	(8.0)
Other	—	0.2
Net cash used in investing activities	(751.5)	(38.1)
Cash flows from financing activities:		
Payments on term loan	(65.3)	(32.7)
Payments on revolving credit facility	—	(250.0)
Repurchases of common stock	(360.8)	(48.9)
Net proceeds from issuance of common stock under share-based compensation arrangements	12.9	20.5
Other	(17.5)	(2.4)
Net cash used in financing activities	(430.7)	(313.5)
Effect of exchange rate changes on cash and cash equivalents and restricted cash	(8.1)	0.7
Net change in cash and cash equivalents and restricted cash	149.3	617.4
Cash and cash equivalents and restricted cash at beginning of period	2,723.6	1,367.3
Cash and cash equivalents and restricted cash at end of period	$ 2,872.9	$ 1,984.7

Reconciliation of GAAP to non-GAAP R&D Expense

	2017	2018	2019	2020
GAAP R&D Expense	$ 736	$ 646	$ 721	$ 948
Share-based compensation	76	57	62	92
Upfront and milestone payments related to licenses and collaborations	49	27	103	-
Restructuring related expenses	16	0	-	-
Non-GAAP R&D Expense	$ 878	$ 730	$ 886	$ 1,040

Reconciliation of GAAP to non-GAAP EPS

	2016	2017	2018	2019	2020
GAAP net income	$ 399.4	$ 443.3	$ 77.6	$ 2,404.3	$ 252.0
Before tax adjustments:					
Cost of sales:					
Share-based compensation	11.1	11.1	16.0	14.2	16.0
Fair value adjustment in inventory acquired	10.8	5.2	-	-	-
Restructruing related expenses	-	152.1	5.8	-	-
Research and development expense:					
Share-based compensation	57.6	76.4	57.4	61.7	92.5
Upfront and milestone payments related to licenses and other strategic agreements	9.6	49.4	26.7	103.4	-
Restructruing related expenses	-	16.3	0.1	-	-
Selling, general and administrative expense:					
Share-based compensation	123.7	155.7	129.6	161.1	180.0
Restructruing related expenses	-	10.9	19.4	-	-
Litigation charges	-	-	13.0	0.1	22.0
Gain on sale of asset	-	-	(3.5)	-	-
Acquired in-process research and development	-	-	1,183.0	(4.1)	-
Amortization of purchased intangible assets	322.2	320.1	320.1	309.6	202.0
Change in fair value of contingent consideration	35.7	41.0	116.5	11.6	31.0
Acquisition-related costs	2.3	-	-	-	131.0
Restructuring expenses	3.0	104.6	25.5	12.0	(1.0)
Impairment of intangible assets	85.0	31.0	-	-	2,053.0
Investment income and (expense):					
(Gains) and losses related to strategic equity investments	-	-	(43.1)	(59.7)	(26.0)
Other income and (expense):					
Gain related to purchase option	-	-	-	(32.0)	-
Restructuring related expenses	-	2.6	(0.1)	-	-
Adjustments to income tax expense	(6.0)	(82.2)	(145.4)	(584.9)	(517.0)
Non-GAAP net income	$ 1,054.4	$ 1,337.5	$ 1,798.6	$ 2,397.3	$ 2,435.5
GAAP earnings per common share - diluted	$ 1.76	$ 1.97	$ 0.35	$ 10.70	$ 1.13
Non-GAAP earnings per common share - diluted	$ 4.62	$ 5.86	$ 7.92	$ 10.53	$ 10.80
Shares used in computing diluted earnings per common share (GAAP)	226.3	225.4	224.5	224.8	222.5
Shares used in computing diluted earnings per common share (non-GAAP)	228.3	228.1	227.1	227.6	225.5

Reconciliation of GAAP to non-GAAP Operating Margin	2017	2018	2019	2020
GAAP operating margin (% of total revenues)	**18%**	**7%**	**42%**	**5%**
Share-based compensation	7%	5%	5%	5%
Amortization of purchased intangible assets	9%	8%	6%	4%
Change in fair value of contingent consideration	1%	3%	0%	1%
Upfront payments related to licenses and other strategic agreements	1%	1%	2%	0%
Contingent milestone payments	0%	0%	0%	0%
Acquired in-process research and development	0%	29%	0%	0%
Acquisition-related cost	0%	0%	0%	2%
Restructuring expenses	8%	1%	0%	0%
Litigation charges	0%	0%	0%	0%
Gain on sale of asset	0%	0%	0%	0%
Impairment of intangible assets	1%	0%	0%	37%
Non-GAAP operating margin (% of total revenues)	**45%**	**53%**	**56%**	**54%**