ALEXION

January 12, 2021

# 39th Annual J.P. Morgan Healthcare Conference

Aradhana Sarin, M.D.
Chief Financial Officer

# Forward Looking Statements

This presentation contains forward-looking statements, including statements related to: the proposed acquisition by AstraZeneca and the anticipated timing of such acquisition; the benefits of the acquisition and the ability of the acquisition to deliver value to shareholders; the ability of AstraZeneca to successfully integrate Alexion's operations, and the ability of AstraZeneca to implement its plans, forecasts and other expectations with respect to Alexion's business after the completion of the proposed acquisition and realize expected synergies; Alexion's anticipated financial results (including short-term guidance and long-range financial guidance), anticipated 2020 revenue, operating margin and non-GAAP EPS, revenue by 2025, our cumulative average growth rate through 2025, and peak revenue from our pipeline beyond 2025 (and all of the assumptions, judgments and estimates related to such anticipated future results); 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); plans to establish 7 blockbuster franchises and the targeted indications in each franchise; 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; our ambition to treat 7,500 neurology patients by 2025; our strategy and ability to grow the ANDEXXA business both in indication and geography; ability to realize continued and sustainable growth in our aHUS franchise and metabolic business; 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 both currently approved and in our pipeline; the Company's goals for 2021 and near term events to support value creation for shareholders; 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 expansion of ULTOMIRIS on direct-to-phase 3 rapid proof of concept trials, plans to further diversify our assets and establish novel platforms and the benefits of those plans); plans for additional formulations of ULTOMIRIS (high concentration and subcutaneous) and the timing for regulatory approval and potential benefits of such formulations; Alexion's ambitions for its portfolio of assets; the anticipated pricing of ULTOMIRIS in PNH and aHUS; ambitions to increase aHUS program; the affected patient populations in the indications we are pursuing; plans to develop and launch ALXN1720; plans for our CSR program; the growth potential and plans for our FcRn program; and continued diversification of the pipeline. 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: the risk that the proposed acquisition of Alexion by Astra Zeneca may not be completed and such failure could negatively affect our stock price and future business and financial results and if the Astra Zeneca merger agreement is terminated, we may be forced to pay a termination fee to Astra Zeneca; the severity of the impact of the COVID-19 pandemic on Alexion's business, including on commercial and clinical development programs; 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 September 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 Financial Guidance for explanations of the amounts adjusted to arrive at non-GAAP net income, non-GAAP and non-GAAP earnings per share amounts for the three and nine month periods ended September 30, 2020 and 2019 and for the projected twelve months ending December 31, 2020.

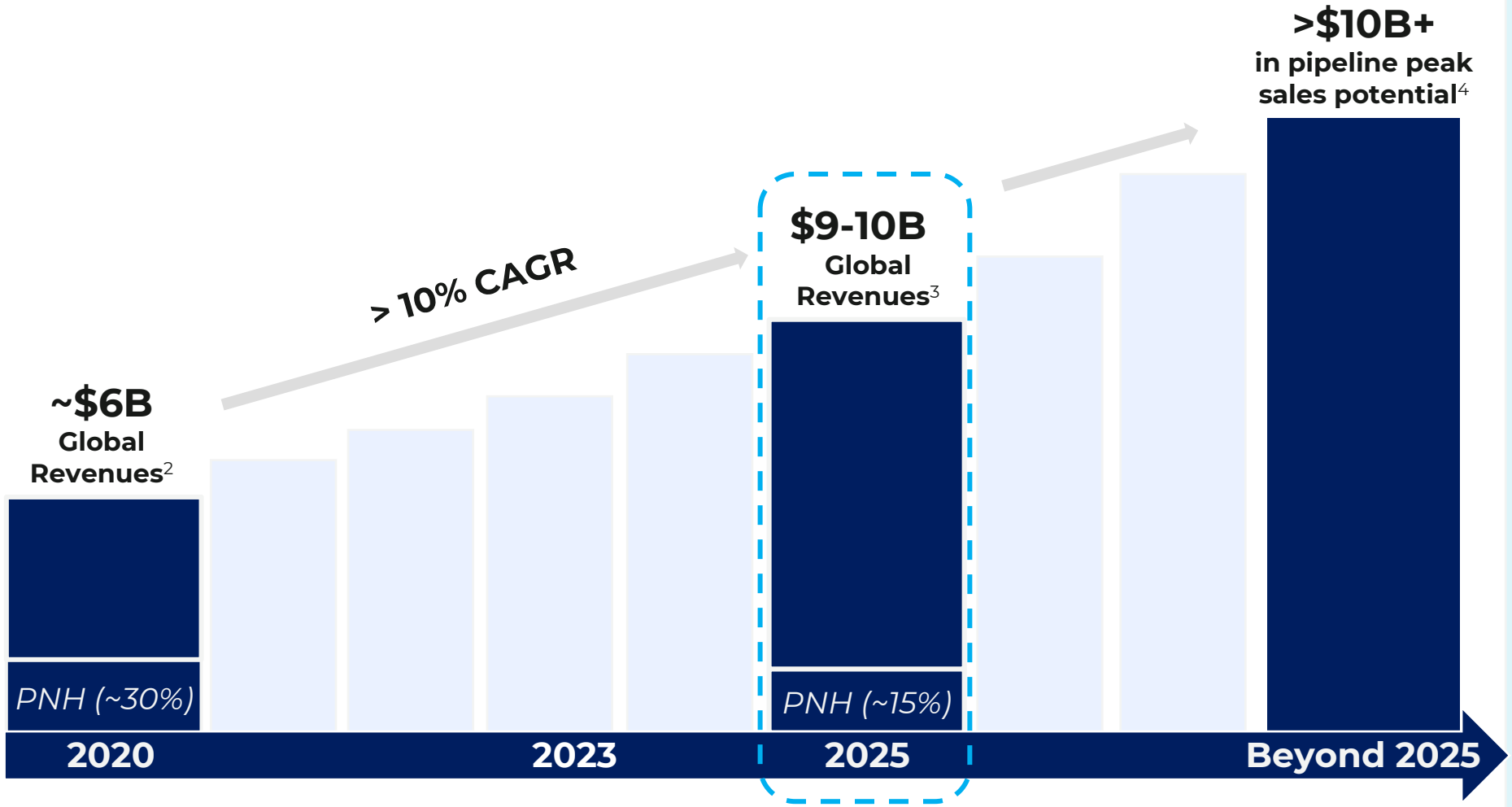
Amounts may not foot due to rounding.

# Our Next Chapter

AstraZeneca & ALEXION

- Advances shared mission of ***following the science*** and using innovative approaches to develop ***life-changing medicines for patients***
- Strengthens AstraZeneca's presence in immunology by adding Alexion's ***strong pipeline*** and ***unique complement technology platforms***
- Combined company to have ***broad global coverage*** across ***primary and specialty care***
- AstraZeneca plans to create ***rare disease business unit***
- Combined organization will be well positioned to ***accelerate innovation and deliver enhanced value*** for our shareholders, patients and rare disease communities we serve

# Standalone ALXN Targeting $9-10B in Global Revenues in 2025




## Key Building Blocks To Achieving 2025 Revenue Ambition

1. **Expand Neurology U.S. Patient Volume 4x** *(to ~7,500 US Patients)*[1]
2. **Grow ANDEXXA Utilization**
3. **Sustainable PNH and Growing aHUS & Metabolic Businesses**
4. **Initial Revenue Contribution From 10 Launches By 2023**

*[1]Ambition Baseline - 12/31/19 1,885 patients [2] 2020 based guidance issued 10/29/20 [3]2025 $9-10B target is at constant currencies (9/30/20 levels) [4] Illustrative, non risk-adjusted revenues, peak sales year varies by program*

# Compelling Portfolio For Patients Today

## 5 Transformative Products

**ULTOMIRIS®**
(RAVULIZUMAB-CWVZ)

**SOLIRIS®**
(ECULIZUMAB)

**STRENSIQ®**
(ASFOTASE ALFA)

**KANUMA®**
(SEBELIPASE ALFA)

**ANDEXXA/ONDEXXYA®**
(ANDEXANET ALFA)

## 7 Rare & Devastating Conditions

**PNH**
(Paroxysmal Nocturnal Hemoglobinuria)

**aHUS**
(Atypical Hemolytic Uremic Syndrome)

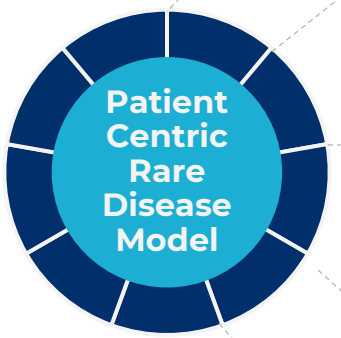
**gMG**
(Generalized Myesthenia Gravis)

**NMOSD**
(Neuromyelitis Optica Spectrum Disorder)

**HPP**
(Hypophosphatasia)

**LAL-D**
(Lysosomal Acid Lipase Deficiency)

**FxA Reversal**
(For Major Life-Threatening Bleeds)

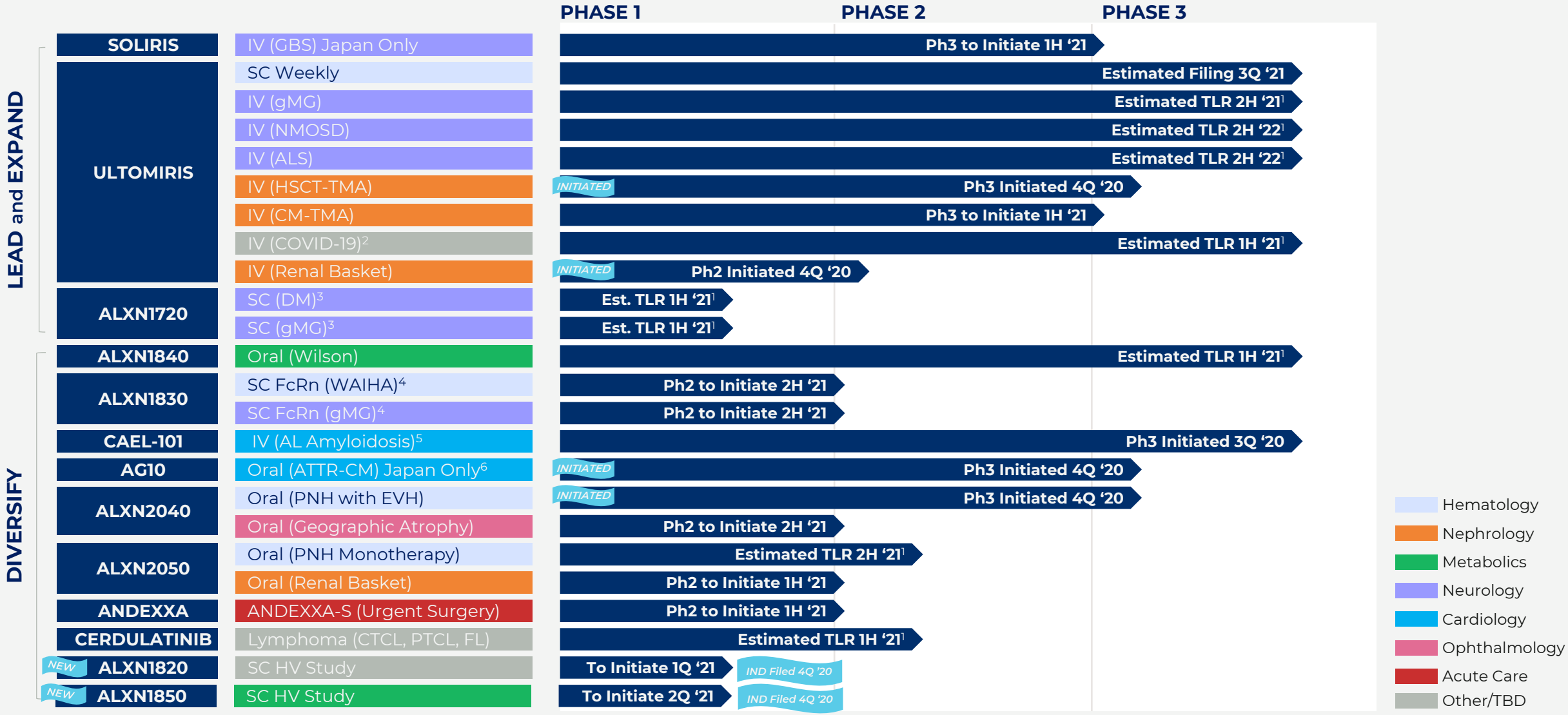



- **Rare Disease Focused Field Force**
- **Best In Class Data Analytics**
- **Operational Excellence In Manufacturing & Supply**
- **Patient-centered Access Models**
- **Personalized High-Touch Patient Support Services***

**RARE DISEASE TAILORED CAPABILITIES, FOCUS & GLOBAL SCALE**

*OneSource is a U.S. Patient Service Model

# Transformed Our Development Pipeline




[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

# With Potential for 7 Blockbuster Franchises

## LEAD AND EXPAND

### Hematology

**Paroxysmal Nocturnal Hemoglobinuria**
(PNH)

**Warm Autoimmune Hemolytic Anemia**
(WAIHA)

### Nephrology

**Atypical Hemolytic Uremic Syndrome**
(aHUS)

**Hematopoietic Stem Cell Transplantation**[2]
(HSCT-TMA)

**Complement Mediated TMA**
(CM-TMA)

**Renal Basket**
(LN, IgAN, PMN, C3G)

### Neurology

**Generalized Myasthenia Gravis**
(gMG)

**Neuromyelitis Optica Spectrum Disorder**
(NMOSD)

**Amyotrophic Lateral Sclerosis**
(ALS)

**Guillain-Barre Syndrome**[1]
(GBS)

**Dermatomyositis**
(DM)

### Metabolics

**Hypophosphatasia**
(HPP)

**Lysosomal Acid Lipase Deficiency**
(LAL-D)

**Wilson Disease**

### Cardiology

**AL Amyloidosis**

**Transthyretin Amyloid Cardiomyopathy**[1]
(ATTR-CM)

### Ophthalmology

**Geographic Atrophy**
(GA)

### Acute Care

**Factor Xa Major Bleeds**

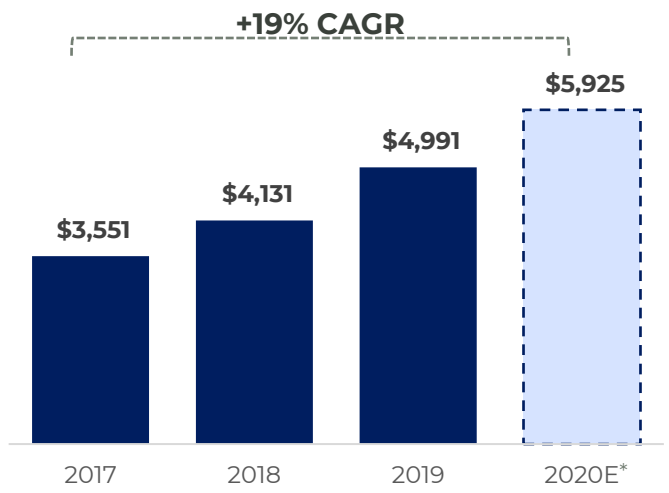
**Factor Xa Reversal for Urgent Surgery**

## DIVERSIFY

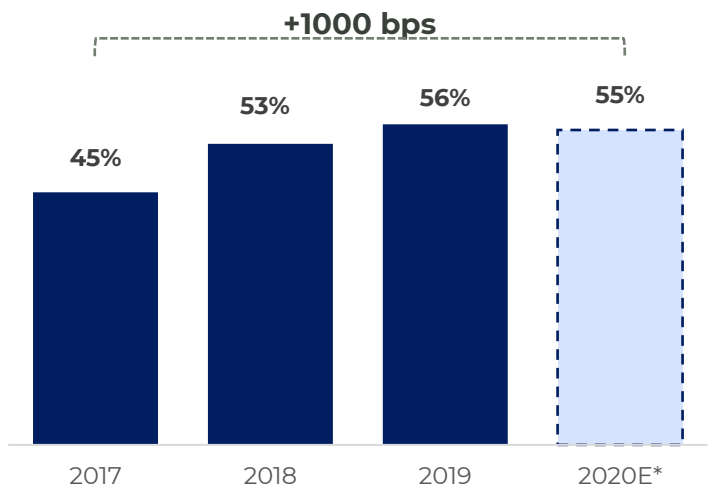
[1]Japan Development Only

# Strong Financial Execution

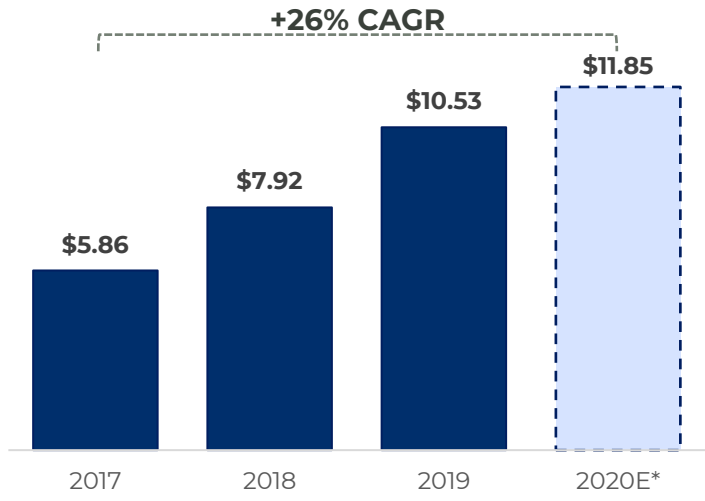
## Double Digit Revenue Growth ($M)




## Best-In-Class Non-GAAP Operating Margins[1]




## Consistent Non-GAAP EPS Growth[1]



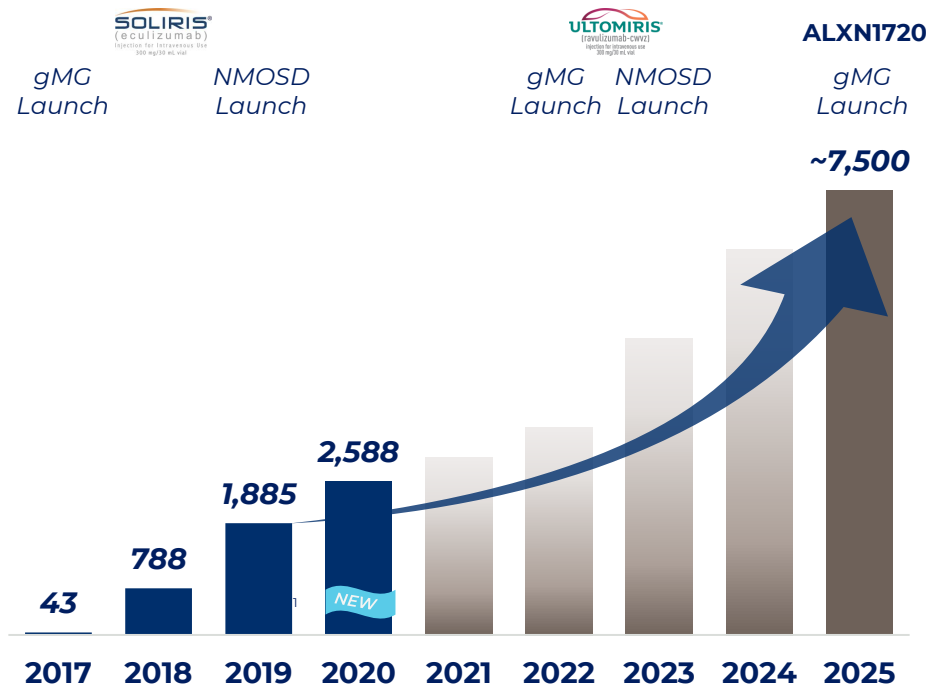

**+19% YOY REVENUE GROWTH VS. 2019 HIGHLIGHTS RESILIENCE OF BUSINESS DESPITE COVID-19**

[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 financial guidance issued on October 29, 2020. The 2020 estimates (and related assumptions) set forth on this slide reflect estimates as of October 29, 2020 and the information on this slide has not been updated to reflect any events subsequent to October 29, 2020..

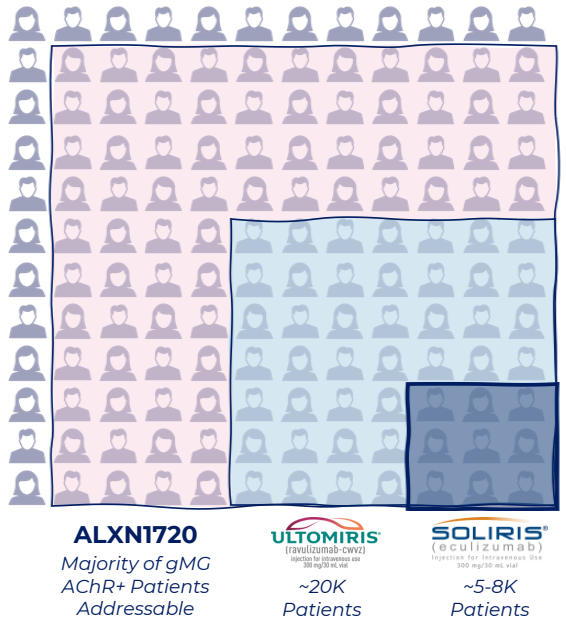
# Neurology is Key Growth Driver through 2025

## AMBITION TO TREAT 4X U.S. NEUROLOGY PATIENTS

### US Neurology Patients (gMG & NMOSD)




### Addressing Incremental Share of gMG Population With ULTOMIRIS Key to Achieving Ambition




*60-80K Total US gMG Patients*

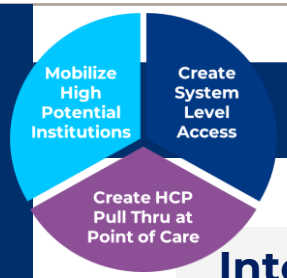
*Estimated ULTOMIRIS launch in gMG 2H 2022*

[1]Ambition Baseline - 12/31/19 1,885 patients (4x growth ambition includes only gMG and NMOSD indications)

# Maximizing ANDEXXA Potential

## Key Progress

- Acceleration of demand to pre-COVID levels in the US
- Filed sBLA to expand US label to include enoxaparin and edoxaban
- Progressing EU payer & access negotiations, launch planning
- Executing against clinical and economic value education plans




## Executing Against Re-Powered Launch Strategy

### Integration and Re-Allocation of Commercial Efforts

Shift field teams to focus towards access and champion mobilization

*Nearing Completion*

### Expand Geographic Reach and Label of ANDEXXA

Seek reimbursement in new markets and pursue development for broader label
(edoxaban/enoxaparin & urgent surgery)

*Underway*

### Focus On Optimizing New and Existing Top Tier Accounts

#### Access Criteria

- Formulary
- Bleeding Protocol
- EMR System Availability[1]
- DUR Conducted[2]

*Underway*

#### Awareness / Advocacy

- Clinical Champions
- Reimbursement Pathway Awareness (incl. NTAP)
- Clinical & Economic Value Education

*Underway*

#### Demand Generation

- Network Center Adoption & Utilization
- Referral Network Activation

*Underway*

[1]EMR: Electronic Medical Record; [2]DUR: Drug Use Review

# Confidence in Sustainability of C5 Franchise

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

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

**ALXN1720**

| ***First Generation C5*** | ***Second Generation C5*** | ***Third Generation C5*** |
|---|---|---|
| *Ultra-Rare Focus*<br>*<6K Patients* | *Expanding to Rare*<br>*>50K Potential Addressable Patients* | *Continued Expansion in Rare*<br>*>100K Potential Addressable Patients* |

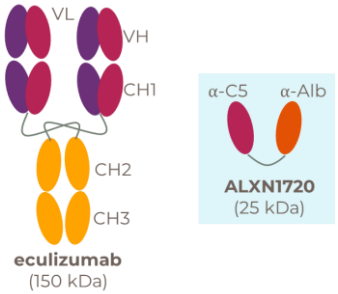
## Compelling ULTOMIRIS Profile

- Majority of C5 market will convert to ULTOMIRIS vs. SOLIRIS
  - Point estimates in favor of ULTOMIRIS on all 11 endpoints across two large Ph3 studies
  - Proven long-term safety record
  - Dosing convenience with only 6-7 (Q8W) 45-minute infusions per year
  - Expected dosing optionality with once-weekly SC self-administration in PNH/aHUS; exploring SC optionality in neurology as clinical data would likely be required
- Convenient product profile offered at a discount annually relative to SOLIRIS
  - Annual treatment cost per patient vs. SOLIRIS is 10% lower in PNH / ~30% lower in aHUS and future Neurology indications in maintenance phase
- Layers of intellectual property protection across indications & geographies

## Bi-Specific Mini-Body

**Long-Acting, Small Volume Subcutaneous Dosing**

*Potential for auto-injector or pre-filled syringe*

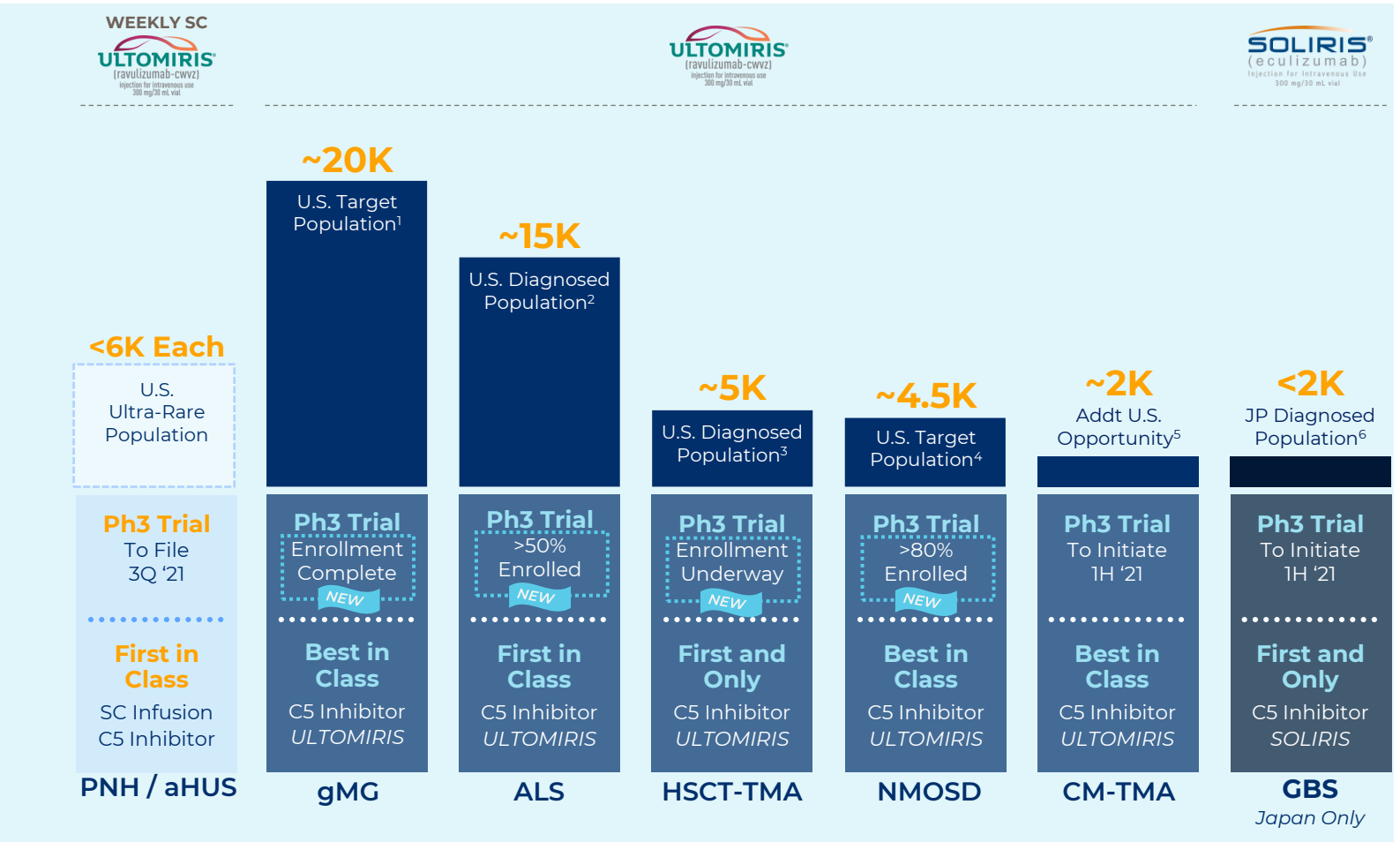



On Track for Ph1 Healthy Volunteer Data 1H 2021

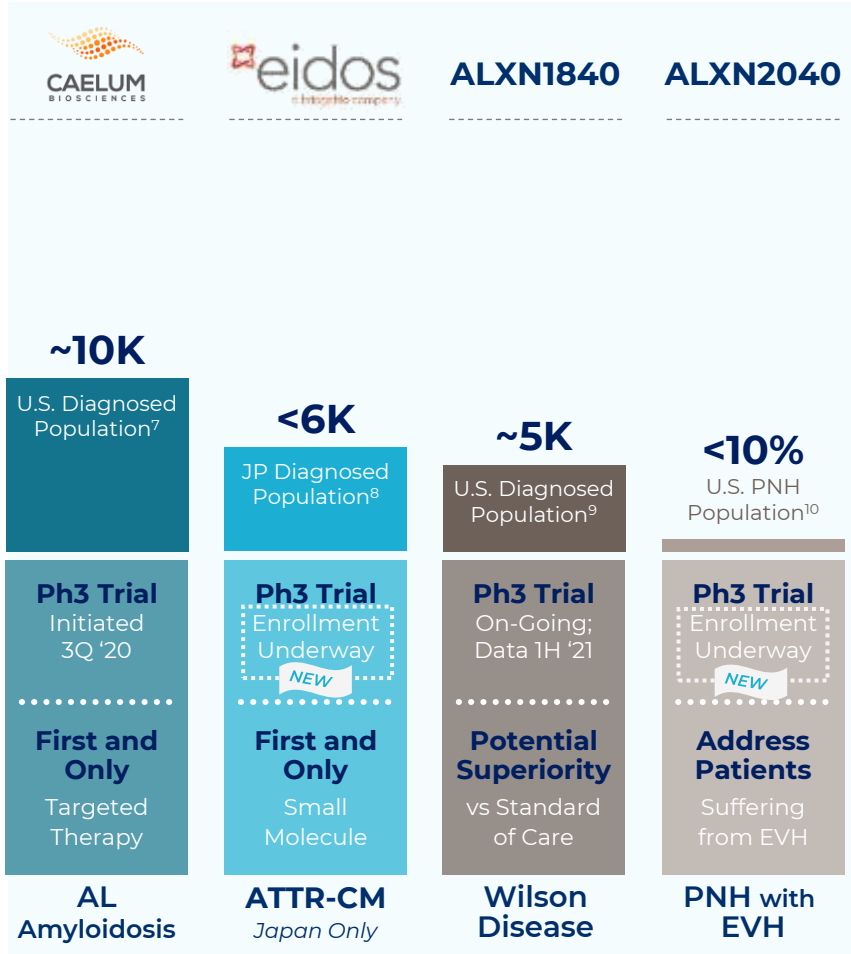
**IMPROVING PROFILE FOR PATIENTS THROUGH THREE GENERATIONS OF C5 INHIBITION**

# On Track For 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

# Advancing Shared Mission to Deliver Life-Changing Medicines

| | 2020 | 2021 |
|---|---|---|
| **LEAD** IN COMPLEMENT | | |
| • Establish ULTOMIRIS as standard of care<br>• Continue to innovate for patients<br>• Develop and launch next generation C5 | ✓ >70% PNH ULTOMIRIS converted in US, DE, JP<br>✓ ULTOMIRIS 100mg/mL approval (US & EU)<br>✓ ALXN1720 Ph1 continued to enroll | ☐ >70% aHUS ULTOMIRIS converted in US (2H)<br>☐ ULTOMIRIS once-weekly SC filing (3Q)<br>☐ ALXN1720 Ph1 top line data (1H) |
| **EXPAND** IN COMPLEMENT | | |
| • Expand presence in Neurology<br>• Focus new ULTOMIRIS expansion on direct to Ph3 and rapid proof of concept studies | ✓ 4x US Neuro ambition set: >700 new patients<br>✓ gMG Ph3 ULTOMIRIS enrollment complete<br>✓ NMOSD Ph3 ULTOMIRIS enrollment >80%<br>✓ ALS Ph3 ULTOMIRIS trial initiated; >50% enrolled | ☐ gMG Ph3 ULTOMIRIS top line data (2H)<br>☐ gMG ULTOMIRIS filing (2H)<br>☐ NMOSD & ALS Ph3 ULTOMIRIS full enrollment (2H)<br>☐ ULTOMIRIS Nephrology[1] enrollment progress (FY) |
| **DIVERSIFY** Into New Growth Areas | | |
| • Expand rare disease focus with novel assets<br>• Grow acute care presence with ANDEXXA | ✓ Ph3 ALXN1840 fully enrolled<br>✓ Ph3 CAEL-101 trial initiated<br>✓ PTLA acquisition closed | ☐ Ph3 ALXN1840 top line data (1H)<br>☐ ALXN1840 filing in Wilson Disease (2H)<br>☐ Ph2 ALXN2040 Geographic Atrophy initiation (2H)<br>☐ ANDEXXA growth (FY) |

**PROPOSED ASTRA ZENECA ACQUISITION OF ALEXION EXPECTED TO CLOSE IN 3Q 2021**

[1] Refers to ULTOMIRIS HSCT-TMA and CM-TMA Ph3 and Renal Basket Ph2 Trials

ALEXION

# Thank You

## 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

## Committed to Corporate Social Responsibility

- **Serve** Communities And Sustain Our Planet
- **Transform** Patient Lives
- **Advance** Our People And Our Company
- **Redefine** What It Means To Live With A Rare Disease

**Ethics & Compliance:** Our Foundation

## Diversity, Inclusion, & Belonging At Alexion

**At Alexion, Diversity is having a seat at the table. Inclusion is having a voice. Belonging is having that voice be heard.**

**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

# Important Additional Information

In connection with the proposed transaction, AstraZeneca PLC ("AstraZeneca") intends to file a registration statement on Form F-4 with the SEC, which will include a document that serves as a prospectus of AstraZeneca and a proxy statement of Alexion Pharmaceuticals, Inc. ("Alexion") (the "proxy statement/prospectus"), Alexion intends to file a proxy statement with the SEC (the "proxy statement") and each party will file other documents regarding the proposed transaction with the SEC. Investors and security holders of Alexion are urged to carefully read the entire registration statement and proxy statement/prospectus or proxy statement and other relevant documents filed with the SEC when they become available, because they will contain important information. A definitive proxy statement/prospectus or a definitive proxy statement will be sent to Alexion's shareholders. Investors and security holders will be able to obtain the registration statement and the proxy statement/prospectus or the proxy statement free of charge from the SEC's website or from AstraZeneca or Alexion as described in the paragraphs below.

The documents filed by AstraZeneca with the SEC may be obtained free of charge at the SEC's website at www.sec.gov. These documents may also be obtained free of charge on AstraZeneca's website at http://www.astrazeneca.com under the tab "Investors".

The documents filed by Alexion with the SEC may be obtained free of charge at the SEC's website at www.sec.gov. These documents may also be obtained free of charge on Alexion's internet website at http://www.alexion.com under the tab, "Investors" and under the heading "SEC Filings" or by contacting Alexion's Investor Relations Department at investorrelations@alexion.com.

## Participants in the Solicitation

Alexion, AstraZeneca, their respective directors and certain of their executive officers and other employees may be deemed to be participants in the solicitation of proxies from Alexion's stockholders in connection with the proposed transaction. Information regarding the persons who may, under the rules of the SEC, be deemed participants in the solicitation of Alexion stockholders in connection with the proposed mergers, including a description of their direct or indirect interests, by security holdings or otherwise, will be set forth in the proxy statement/prospectus when it is filed with the SEC. Information about Alexion's directors and executive officers is available in Alexion's proxy statement for its 2020 annual meeting of stockholders, which was filed with the SEC on March 26, 2020, Alexion's Annual Report on Form 10-K for the fiscal year ended December 31, 2019, which was filed with the SEC on February 4, 2020, and other documents subsequently filed by Alexion with the SEC. Information about AstraZeneca's directors and executive officers is available in AstraZeneca's Form 20-F filed with the SEC on March 3, 2020, and other documents subsequently filed by AstraZeneca with the SEC.

## No Offer or Solicitation

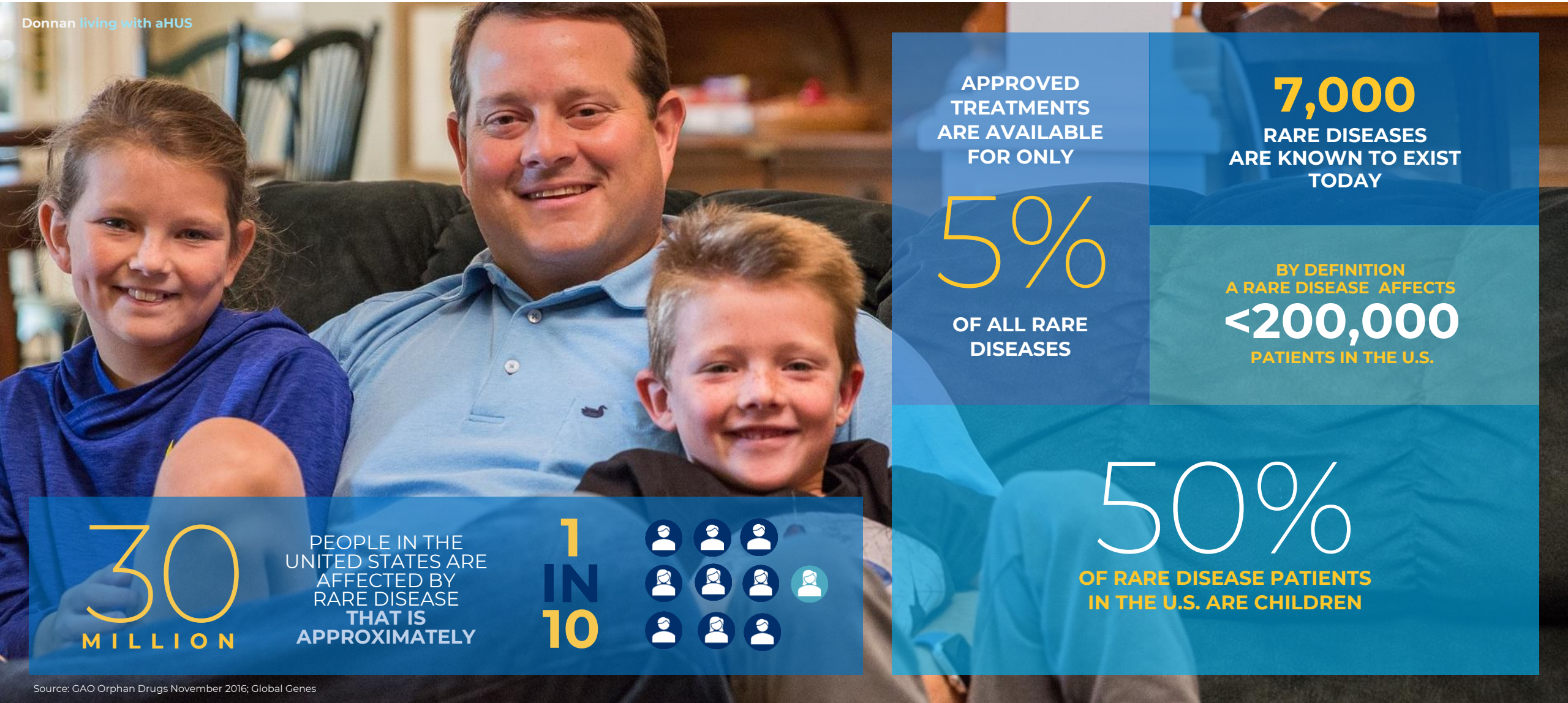
This communication is not intended to and shall not constitute an offer to buy or sell or the solicitation of an offer to buy or sell any securities, or a solicitation of any vote or approval, nor shall there be any sale of securities in any jurisdiction in which such offer, solicitation or sale would be unlawful prior to registration or qualification under the securities laws of any such jurisdiction. No offering of securities shall be made, except by means of a prospectus meeting the requirements of Section 10 of the U.S. Securities Act of 1933, as amended.

Investor day

# APPENDIX


**Jesse** living with gMG

# 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**

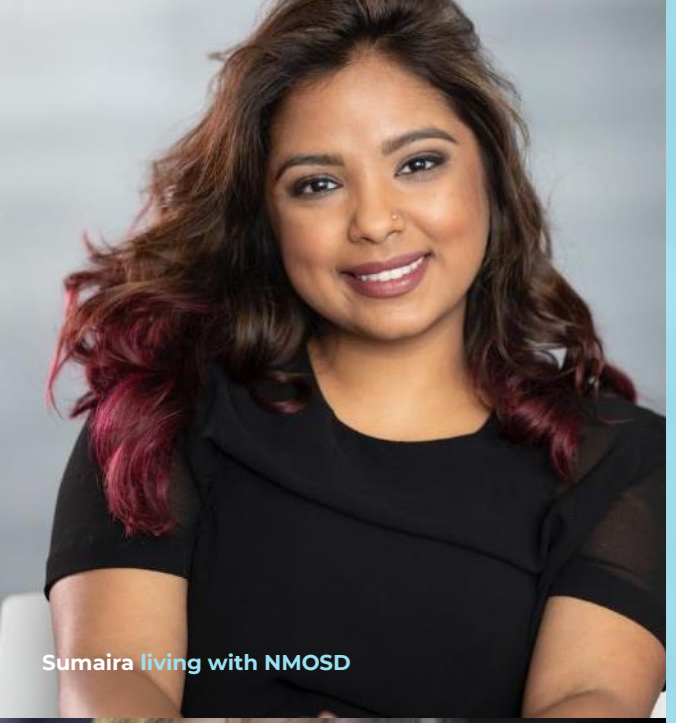
PEOPLE IN THE UNITED STATES ARE AFFECTED BY RARE DISEASE **THAT IS APPROXIMATELY**

**1 IN 10**

**50%**

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

Source: GAO Orphan Drugs November 2016; Global Genes


Sumaira living with NMOSD

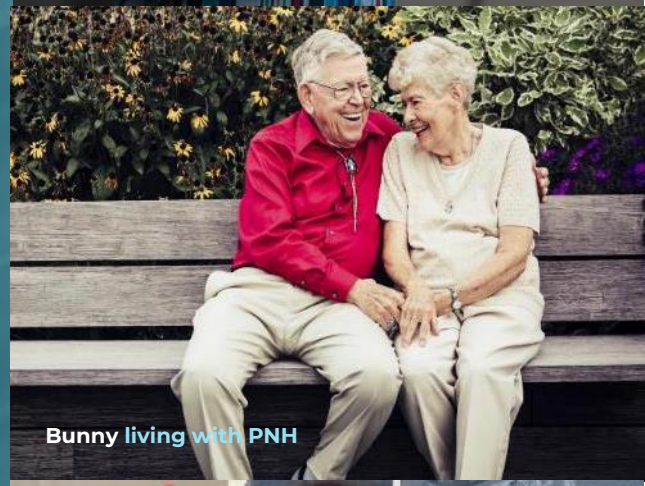

Albie living with LAL-D

# 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

# 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

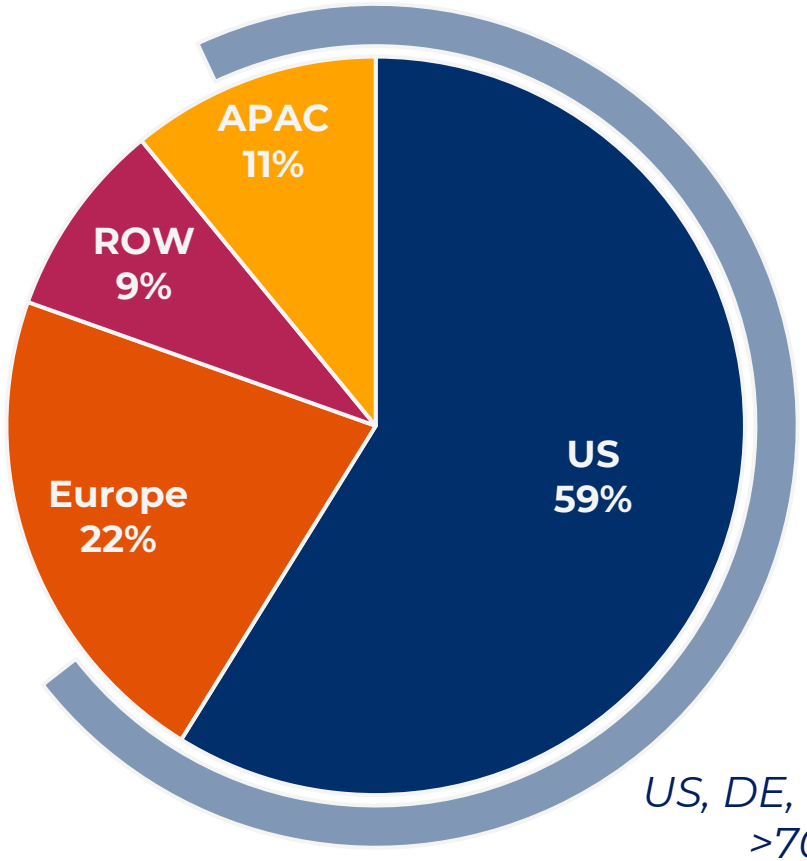
### DIVERSIFY

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

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

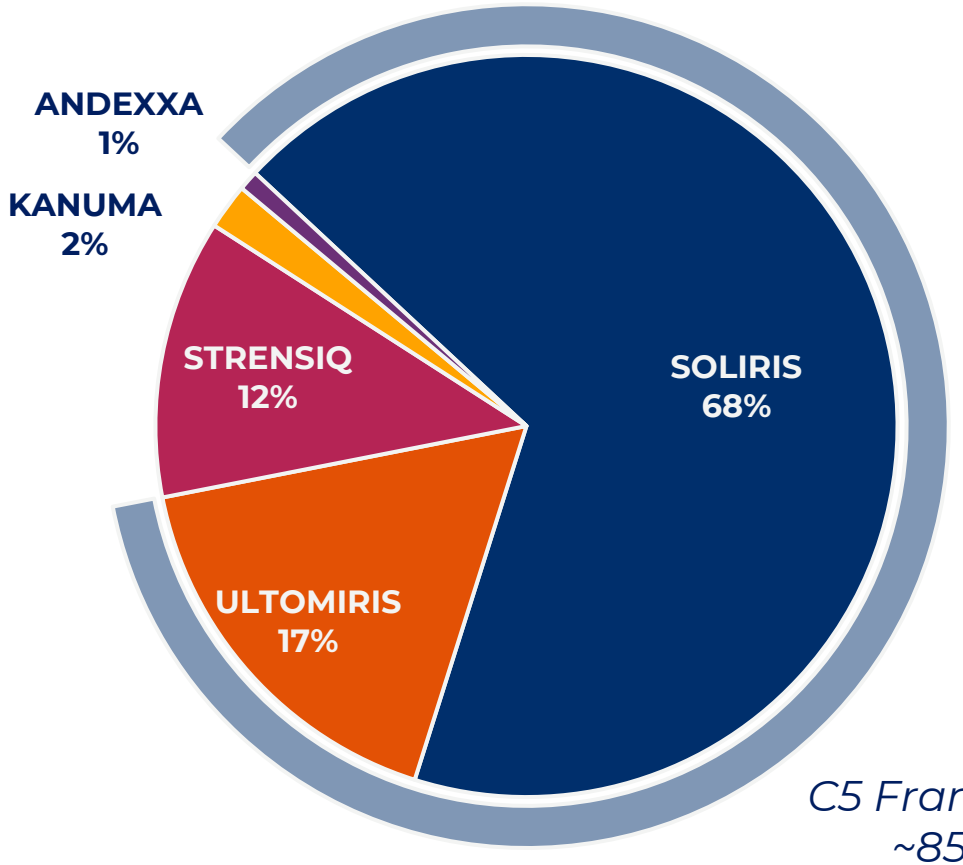
# Q3 2020 YTD Revenue Composition

## Geography




*US, DE, and JP*
*>70%*
*of Global Revenues*

## Product




*C5 Franchise*
*~85%*
*of Global Revenues*

* Through 9/30/2020, as reported 10/29/2020

# ULTOMIRIS Conversion Progress

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

**3Q 2020**

**PNH ULTOMIRIS 70% Conversion In Top Three Markets Achieved**

**aHUS ULTOMIRIS Conversion On Track T0 70% Ambition[1]**

**Dec 2018**
U.S. PNH ULTOMIRIS Approval

**Jun 2019**
JP PNH ULTOMIRIS Approval

**Jul 2019**
EU PNH ULTOMIRIS Approval

**Oct 2019**
U.S. aHUS ULTOMIRIS Approval

**Jun 2020**
EU aHUS ULTOMIRIS Approval

**Sep 2020**
JP aHUS ULTOMIRIS Approval

**2H 2022**
Estimated gMG ULTOMIRIS Approval[2]

**1H 2023**
Estimated NMOSD ULTOMIRIS Approval[2]

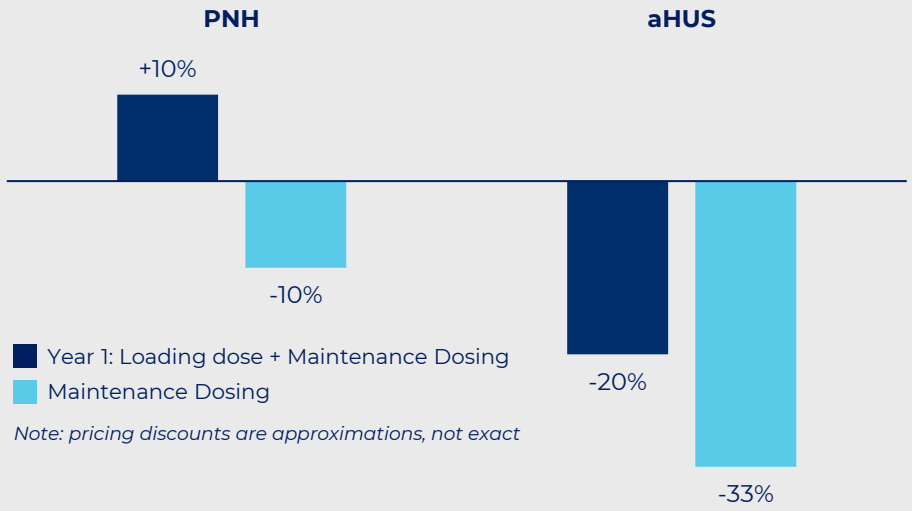
**AMBITION FOR BEST-IN-CLASS CONVERSION ACROSS ALL INDICATIONS**

[1]aHUS ambition of 70% of total patients on ULTOMIRIS within 2 years of launch; [2]Pending regulatory approval following completion of Phase 3 studies

# 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

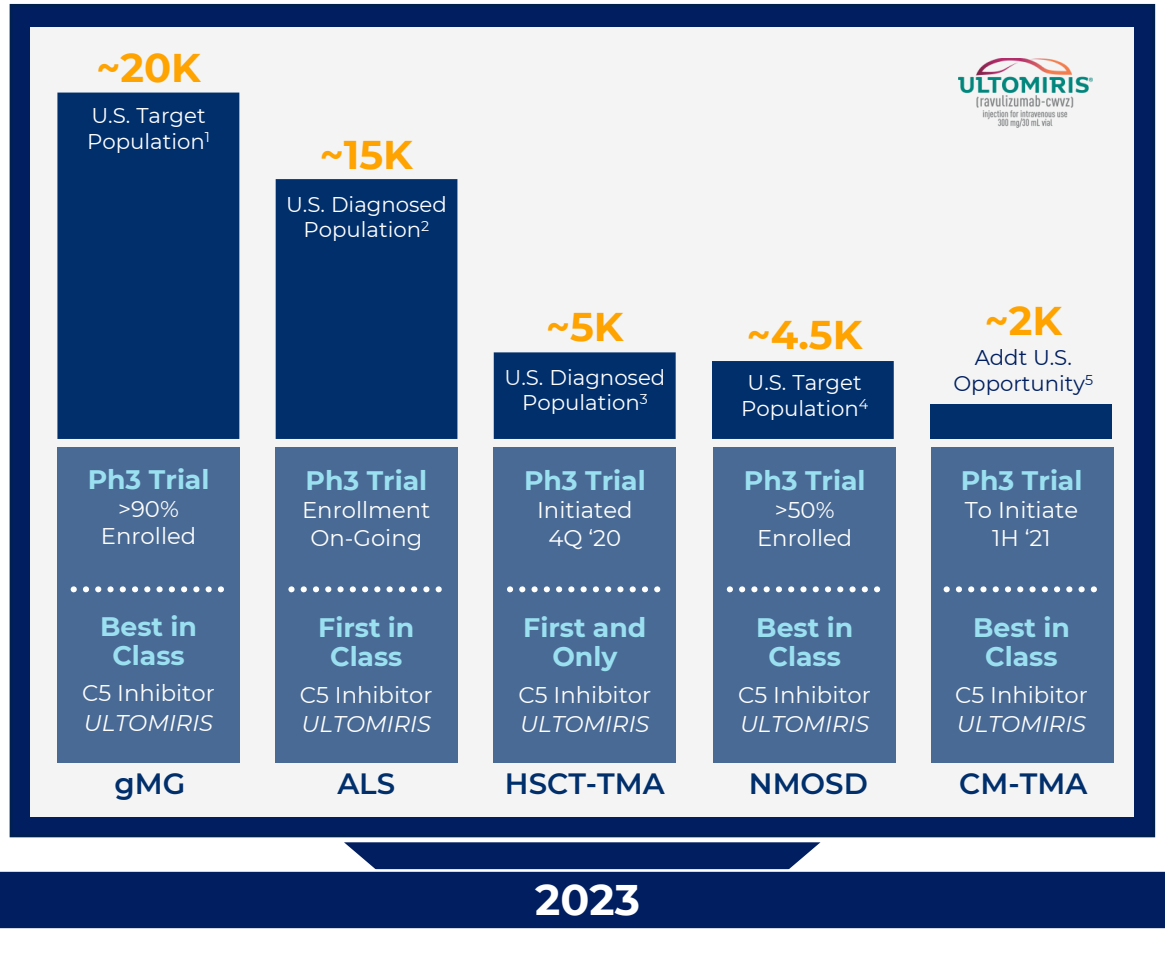
**Patient Sample 1**: Loading dose + 2 Maintenance Infusions


**Patient Sample 2**: Loading dose + 1 Maintenance Infusion


- 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

# Ample Opportunity to Expand C5 Platform Reach

*ULTOMIRIS expansion a key component of ambition for 10 launches by 2023*




*With even broader rare diseases populations in scope for development beyond*

**ULTOMIRIS**

**Renal Basket**

- Pursing role of terminal complement inhibition in IgAN and LN
- US diagnosed prevalence of ~150K patients
- **Ph2 proof of concept trial initiated 4Q 2020**

**ALXN1720**

**gMG**

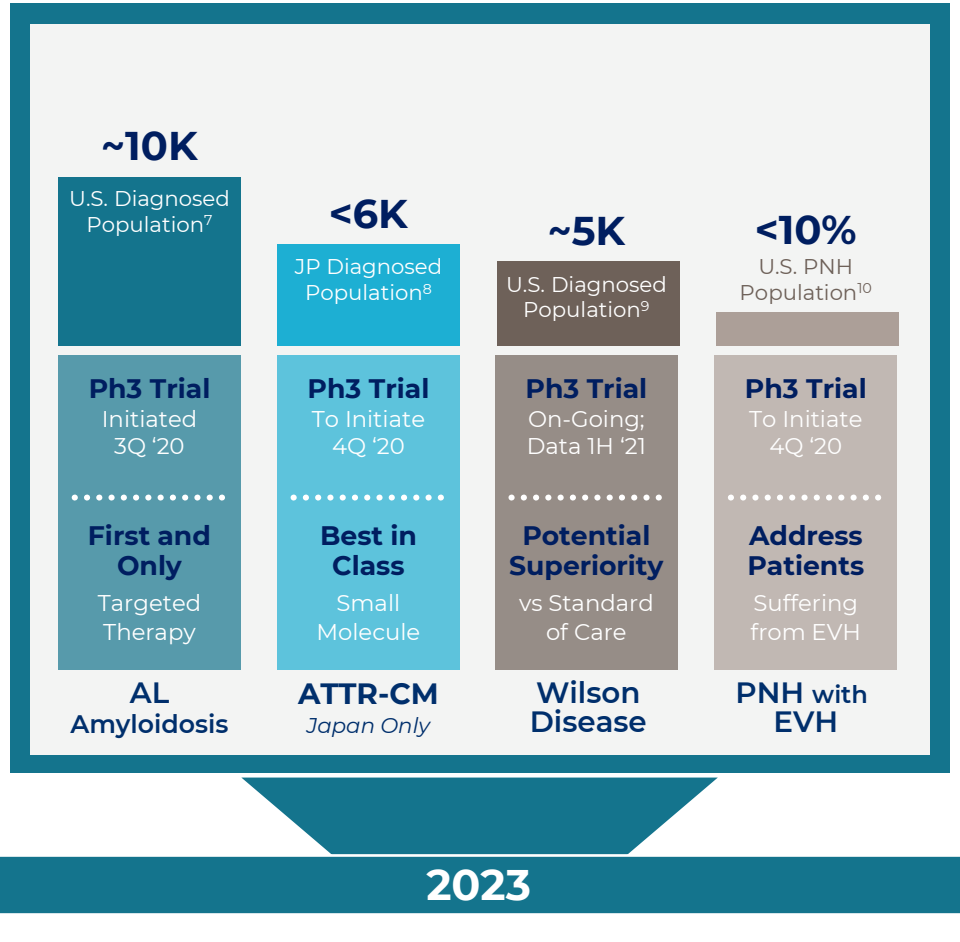
- Evolving gMG portfolio strategy to target the majority of AChR+ gMG patients
- US diagnosed prevalence of 60-80K patients
- **To pursue following Ph1 data in 1H 2021**

**DM**

- Expanding footprint in neuromuscular and rare autoimmune diseases
- US diagnosed prevalence of classic DM ~40K
- **To pursue following Ph1 data in 1H 2021**

# Diversifying Beyond C5

*Opportunities to diversify broaden ambition for 10 launches by 2023*




*With innovative platforms and novel assets continuing to diversify portfolio long-term*

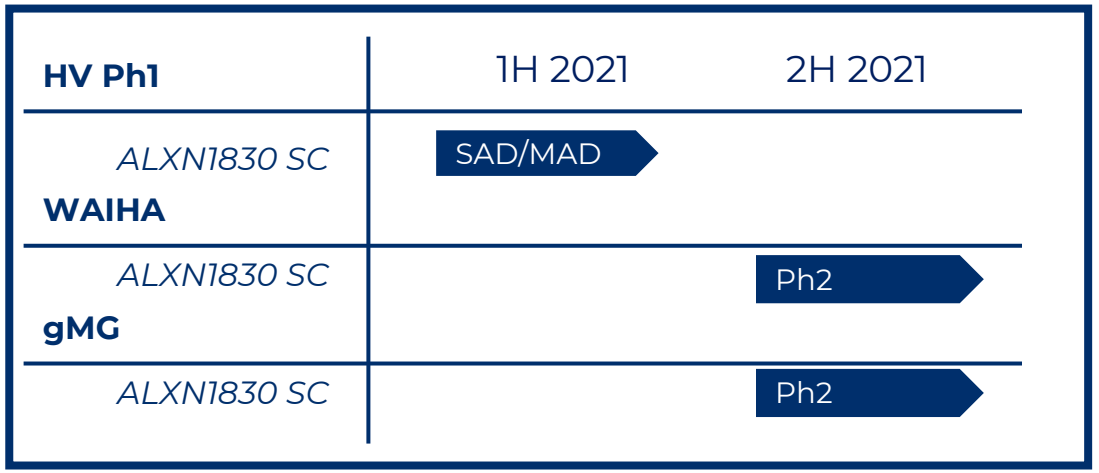
| | | |
|---|---|---|
| ALXN1830 | gMG | • Once-weekly SC FcRn supports gMG portfolio strategy<br>• **Ph2 trial to initiate 2H 2021** |
| | WAIHA | • Once-weekly SC FcRn expands hematology presence<br>• **Ph2 trial to initiate 2H 2021** |
| Factor D | GA | • Systemic, oral approach to slow disease progression<br>• **Ph2 trial to initiate 2H 2021 with ALXN2040** |
| | Renal Basket | • Exploring fD in LN, IgAN, PMN, and C3G<br>• **Ph2 trial to initiate 1H 2021 with ALXN2050** |
| ANDEXXA | | • Launch "reboot" and label expansion efforts underway<br>• **Ph2 Urgent Surgery trial to begin 2H2021** |
| ALXN1820 | | • Novel anti-properdin mini-body<br>• **First-in-human studies to begin 1H 2021** |
| ALXN1850 | | • Next-gen asfotase alfa (STRENSIQ)<br>• **First-in-human studies to begin 1H2021** |

# Vast Opportunity In FcRn Landscape

## ALXN1830 Value Proposition

- Rapid onset of action and sustained IgG lowering after a single dose
- Excellent PK/PD profile for indications of interest with >70% IgG lowering expected and high specificity to IgG
- Reduces IgG immunocomplexes levels
- Superior dosing profile with once weekly subcutaneous administration
- Favorable safety profile to date:
  - No effect on albumin, eliminating concerns of hypoalbuminemia
  - No headache seen thus far in SC HV
- Potential for combination therapy with Alexion's complement mini-bodies including ALXN1720 and ALXN1820

***FcRn Has Potential to Treat Hundreds of Thousands of Patients with IgG Mediated Diseases Including gMG, WAIHA, CIDP etc***




## 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**

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

## *LEAD* and *EXPAND* in complement

**<$100M**
**SOLIRIS**
*GBS (2023)*
*Japan Only*

**$1 to $4B**
**ULTOMIRIS**
**New Indications**
*ALS (2023)*
*HSCT-TMA (2023)*
*CM-TMA (2023)*

**$2 to $3B**
**ALXN1720**
*DM (2023+)*
*gMG (2023+)*

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

**$500M to $1B**
**ALXN1840**
*Wilson Disease (2022)*

**$1.0B+**
**ANDEXXA**
*Factor Xa (2020)*
*Urgent Surgery (2023+)*

**$1.0B+**
*Combined*
**CAEL-101**[1]
*AL Amyloidosis (2023)*
**AG10**
*ATTR-CM (2023)*
*Japan Only*

**$1.0B+**
**ALXN1830**
*WAIHA (2023+)*
*gMG (2023+)*

**$500M to $2.0B+**
**Factor D**[2]
*PNH (2023+)*
*GA (2023+)*
*Renal (2023+)*

**7 Blockbuster Franchises**

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

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

# Near-Term Events Support Alexion's Value Creation Strategy

| | | | |
|---|---|---|---|
| **LEAD** | US IPR Settlement (SOLIRIS Patents) | 2Q 2020 | ☑ |
| | ULTOMIRIS PNH Subcutaneous Ph3 Top Line Results (PK) | 2Q 2020 | ☑ |
| | ULTOMIRIS aHUS EMA Approval by EC | Mid 2020 | ☑ |
| | ULTOMIRIS 100mg/ml Formulation FDA & EMA 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 Interim Results | 1H 2021 | ☐ |
| | ULTOMIRIS gMG Ph3 Top Line Results | 2H 2021 | ☐ |
| | ULTOMIRIS ALS Ph3 Top Line Results | 2H 2022 | ☐ |
| | ULTOMIRIS NMOSD Ph3 Top Line Results | 2H 2022 | ☐ |
| | ULTOMIRIS gMG FDA Approval | 2H 2022 | ☐ |
| **DIVERSIFY** | Portola Acquisition Close | 3Q 2020 | ☑ |
| | ALXN2040 C3G Ph2 Top Line Results | Mid 2020 | ☑ |
| | ALXN2060 (AG10) Japan Ph3 Initiation | 4Q 2020 | ☑ |
| | CAEL-101 Ph3 Trial Initiation | 2H 2020 | ☑ |
| | ALXN1820 IND Filing | 2H 2020 | ☑ |
| | ALXN1850 IND Filing | 2H 2020 | ☑ |
| | ALXN1840 Wilson Ph3 Top Line Results | 1H 2021 | ☐ |
| | ALXN2050 PNH Ph2 Top Line Results | 2H 2021 | ☐ |
| | ALXN2040 GA Ph2 Initiation | 2H 2021 | ☐ |
| | ALXN2060 (AG10) Japan Ph3 Top Line Results | 2H 2022 | ☐ |
| | ALXN1840 Wilson Launch | 2H 2022 | ☐ |

# CSR and ESG at 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

*"At Alexion, we work to change lives for the better – ours, people living with rare diseases and the communities we serve – and our commitment to being a responsible corporate citizen helps make it possible."*

**CEO LUDWIG HANTSON**

## Recognition *(Alexion's Inaugural CSR Report Published in 2020)*

Corporate ESG Performance – Prime – RATED BY ISS ESG

1st Decile Rank (Pharmaceuticals & Biotech)

S&P Global Ratings

Double Digit Growth

SUSTAINALYTICS a Morningstar company – ESG INDUSTRY TOP RATED 2020 2021

#1 ESG Risk Rating (Biotech)

America's Most Responsible Companies 2021 – Newsweek – statista

Ranked 161 out of 400 (Top 40%)

**COMMITTED TO CONTINUING ELEVATION OF CSR REPORTING IN 2021**

Download Alexion's Inaugural 2019 CSR Report at csr.alexion.com

# Commercial Portfolio Patent & Orphan Exclusivity

| Product | Region | Patent Exclusivity | Orphan Exclusivity | Data Exclusivity |
|---|---|---|---|---|
| **ULTOMIRIS** | US | 2035 | PNH 2025<br>aHUS (SC only; filing 3Q 2021) | 2030 |
| | EU | 2035 | N/A | 2029 |
| | Japan | 2035 | PNH 2029 | 2029 |
| **SOLIRIS** | US | 2027[1] | gMG 2024<br>NMO 2026 | 2019 |
| | EU | 2020[2] | aHUS 2023<br>gMG 2027<br>NMO 2029 | 2018 |
| | Japan | 2027 | gMG 2027<br>NMO 2029 | 2020 |
| **STRENSIQ** | US | 2029 | 2022 | 2027 |
| | EU | 2030 | 2027 | 2025 |
| | Japan | 2028 | 2025 | 2025 |
| **KANUMA** | US | 2031 | 2022 | 2027 |
| | EU | 2031 | 2027 | 2025 |
| | Japan | 2031 | 2026 | 2026 |
| **ANDEXXA/ONDEXXYA** | US | 2030 | 2025 | 2030 |
| | EU | 2033 | N/A | 2029 |
| | Japan | 2028 | N/A | |

[1] Alexion licensed Amgen to commercialize biosimilar eculizumab effective March 1, 2025 (or earlier in certain circumstances). See IPR settlement agreement dated May 28, 2020
[2] The following patents are under appeal which would extend patent to 2027: '834 Method of Use Patent was approved, then subsequently revoked in January 2019. Patent is in effect as Alexion appeals. '888 and '029 patent applications were rejected, and Alexion has begun the process to appeal these decisions. These patents are not in effect during appeal

| **Identifier** (Other) | **Name** (INN) | **MOA** | **ROA** | **Indication** | **Phase** | **Study Start** | **Anticipated Study End** |
|---|---|---|---|---|---|---|---|
| **SOLIRIS** | (eculizumab) | Anti-C5 | Q2W IV | Guillain Barre Syndrome | Ph3 | Initiating 1H '21 | Not yet disclosed |
| **ALXN1210** | **ULTOMIRIS** (ravulizumab) | Anti-C5 | Q1W SC | Paroxsymal Nocturnal Hemoglobinuria (PNH)<br>Atypical Hemolytic Uremic Syndrome (aHUS) | Ph3 | Initiated 1Q '19 | TLR 2Q '20<br>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 2H '22 |
| | | | | Amyotrophic Lateral Sclerosis (ALS) | Ph3 | Initiated 1Q '20 | TLR 2H '22 |
| | | | | Hematopoetic Stem Cell Transplant Thrombotic Microangiopathy (HSCT-TMA) | Ph3 | Initiated 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 | Initiated 4Q '20 | Not yet disclosed |
| **ALXN1720** | **N/A** | Anti-C5 Bi-Specific | SC | Generalized Myasthenia Gravis (gMG)[1] | Ph1 HV | Reinitiated 3Q '20 | TLR 1H '21 |
| | | | | Dermatomyositis (DM)[1] | | | |
| **ALXN1840** *(WTX-101)* | *(Bis-choline tetrathiomolybdate)* | Copper chelator | Oral | Wilson Disease | Ph3 | Initiated 1Q '18 | TLR 1H '21 |
| **ALXN1830** *(SYNT-001)* | **N/A** | Anti-FcRn | SC | Warm Autoimmune Hemolytic Anemia (WAIHA)[2] | Ph1 HV | Reinitiating 1H '21 | TLR 1H '21 |
| | | | | Generalized Myasthenia Gravis (gMG)[2] | | | |
| **CAEL-101** | **N/A** | ALκ/ALλ fibril reactive antibody | IV | Amyloid Light-Chain (AL) Amyloidosis | Ph3 | Initiated 3Q '20 | TLR 2H '22 |
| **ALXN2060** *(AG10)* | (acoramidis) | TTR tetramers stabilizer (small molecule) | Oral | Transthyretin Amyloid Cardiomyopathy (ATTR-CM) | Ph3 | Initiated 4Q '20 | TLR 2H '22 |
| **ALXN2040** *(ACH-4471)* | (danicopan) | Factor D inhibitor (small molecule) | TID Oral | PNH with Extravascular Hemolysis (PNH w/ EVH) | Ph3 | Initiated 4Q '20 | TLR 2H '22 |
| | | | TBD | Geographic Atrophy | Ph2 | Initiating 2H '21 | Not yet disclosed |
| **ALXN2050** *(ACH-5228)* | (vermicopan) | 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 |
| **ALXN2070** | **ANDEXXA** (andexanet alfa) | Factor Xa Reversal | IV | Urgent Surgery | Ph2 | Initiating 1H '21 | Not yet disclosed |
| **ALXN2075** | (cerdulatinib) | SYK/JAK kinase inhibitor | Oral | Lymphoma (CTCL, PTCL, FL) | Ph2 | PTLA Acquisition | TLR 1H '21 |
| **ALXN1820** | **N/A** | Anti-Properdin Mini-Body | SC | Not yet disclosed | Ph1 | Initiating 1Q '21 | Not yet disclosed |
| **ALXN1850** | **N/A** | Next generation alfotase alfa | SC | Not yet disclosed | Ph1 | Initiating 2Q '21 | Not yet disclosed |

[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

# 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. | |

| | 2017 | 2018 | 2019 | 2020E* |
|---|---|---|---|---|
| **GAAP operating margin (% of total revenues)** | **18%** | **7%** | **42%** | **8%** |
| Share-based compensation | 7% | 5% | 5% | 4% |
| 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% | 35% |
| Fair value adjustment in inventory acquired | 0% | 0% | 0% | 0% |
| **Non-GAAP operating margin (% of total revenues)** | **45%** | **53%** | **56%** | **55%** |

**2020E based on midpoint of 2020 Guidance issued October 29, 2020*

## Reconciliation of GAAP to non-GAAP EPS

| | 2016 | 2017 | 2018 | 2019 | 2020E* |
|---|---|---|---|---|---|
| **GAAP net income** | $ 399.4 | $ 443.3 | $ 77.6 | $ 2,404.3 | $ 434.0 |
| Before tax adjustments: | | | | | |
| Cost of sales: | | | | | |
| Share-based compensation | 11.1 | 11.1 | 16.0 | 14.2 | 13.5 |
| Fair value adjustment in inventory acquired | 10.8 | 5.2 | - | - | 23.0 |
| Restructruing related expenses | - | 152.1 | 5.8 | - | - |
| Research and development expense: | | | | | |
| Share-based compensation | 57.6 | 76.4 | 57.4 | 61.7 | 72.5 |
| 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 | - | - |
| Fair value adjustment in inventory acquired | - | - | - | - | 1.00 |
| Selling, general and administrative expense: | | | | | |
| Share-based compensation | 123.7 | 155.7 | 129.6 | 161.1 | 165.0 |
| Restructruing related expenses | - | 10.9 | 19.4 | - | - |
| Litigation charges | - | - | 13.0 | 0.1 | 22.0 |
| Gain on sale of asset | - | - | (3.5) | - | (15.0) |
| Acquired in-process research and development | - | - | 1,183.0 | (4.1) | - |
| Amortization of purchased intangible assets | 322.2 | 320.1 | 320.1 | 309.6 | 254.0 |
| Change in fair value of contingent consideration | 35.7 | 41.0 | 116.5 | 11.6 | 51.0 |
| Acquisition-related costs | 2.3 | - | - | - | 120.0 |
| Restructuring expenses | 3.0 | 104.6 | 25.5 | 12.0 | 25.0 |
| 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) | (34.0) |
| 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) | (518.5) |
| **Non-GAAP net income** | $ 1,054.4 | $ 1,337.5 | $ 1,798.6 | $ 2,397.3 | $ 2,666.5 |
| | | | | | |
| **GAAP earnings per common share - diluted** | $ 1.76 | $ 1.97 | $ 0.35 | $ 10.70 | $ 1.96 |
| **Non-GAAP earnings per common share - diluted** | $ 4.62 | $ 5.86 | $ 7.92 | $ 10.53 | $ 11.85 |
| | | | | | |
| **Shares used in computing diluted earnings per common share (GAAP)** | 226.3 | 225.4 | 224.5 | 224.8 | 222.0 |
| **Shares used in computing diluted earnings per common share (non-GAAP)** | 228.3 | 228.1 | 227.1 | 227.6 | 225.0 |

**2020E based on midpoint of 2020 Guidance issued October 29, 2020*