

UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT  
PURSUANT TO SECTION 13 OR 15(D) OF  
THE SECURITIES EXCHANGE ACT OF 1934

Date of report (Date of earliest event reported): January 13, 2020

**ALEXION PHARMACEUTICALS, INC.**

(Exact name of registrant as specified in its charter)

Delaware

000-27756

13-3648318

(State or other jurisdiction  
of incorporation or organization)

(Commission  
File Number)

(I.R.S. Employer  
Identification No.)

121 Seaport Boulevard, Boston, Massachusetts 02210

(Address of Principal Executive Offices) (Zip Code)

Registrant's telephone number, including area code: (475) 230-2596

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value \$0.0001 per share	ALXN	Nasdaq Global Select Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging Growth Company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

**Item 2.02. Results of Operations and Financial Condition.**

Please see the disclosure relating to the estimated revenue growth for Alexion Pharmaceuticals, Inc. (“Alexion” or the “Company”) in 2019, set forth under Item 7.01 “Regulation FD Disclosure” of this Current Report on Form 8-K, which is incorporated by reference into this Item 2.02.

**Item 7.01 Regulation FD Disclosure.**

Alexion Pharmaceuticals, Inc. (“Alexion” or the “Company”) will participate in the 38th Annual J.P. Morgan Healthcare Conference in San Francisco, California on January 13-16, 2020. Alexion Chief Executive Officer, Ludwig Hantson, will make a presentation on Tuesday, January 14th, at 10:30 a.m. ET/7:30 a.m. PT using the slides attached to this Current Report on Form 8-K as Exhibit 99.1 (the “Conference Presentation”) and incorporated herein by reference. The presentation will be webcast live and will be available at <http://ir.alexion.com> by clicking on an available link.

We expect that Mr. Hantson will discuss during the presentation and the question and answer session, among other things, the following matters:

- Two new additional development programs (ULTOMIRIS IV(CM-TMA) and ALXN1810 Renal Basket);
- As of December 31, 2019, SOLIRIS NMOSD and gMG patients in the United States totaled 1,885;
- Alexion achieved greater than 20% revenue growth in 2019 as compared to 2018 (this amount is a preliminary estimate and is subject to completion of the audit of the income statement for the fiscal year ended December 31, 2019);
- Updated ULTOMIRIS PNH patient conversion numbers (as of January 10, 2020);
- Timing of anticipated future product launches and clinical trials;
- Anticipated neurology patients on Alexion products by 2025;
- Information regarding the expected clinical trial of ULTOMIRIS for ALS;
- Revenue attributable to sales of metabolic products in 2019 (this amount is a preliminary estimate and is subject to completion of the audit of the income statement for the fiscal year ended December 31, 2019); and
- The deadline established in our by-laws for shareholders to nominate directors for consideration at the 2020 annual meeting has passed and we did not receive any director nominations from shareholders.

The information in this Current Report on Form 8-K and the attached Conference Presentation that we expect will be utilized at the 38th Annual J.P. Morgan Healthcare Conference, and the information set forth therein, is being furnished pursuant to Item 2.02 and Item 7.01 of this Current Report on Form 8-K and shall not be deemed “filed” for purposes of Section 18 of the Securities Exchange Act of 1934 (the “Exchange Act”) or otherwise subject to the liabilities of that Section. Nor shall such documents or information be deemed incorporated by reference in any filing under the Securities Act of 1933 or the Exchange Act, regardless of any general incorporation language in the filing unless specifically stated so therein.

**Item 9.01 Financial Statements and Exhibits.**

(d) Exhibits.

Exhibit Number

Description

99.1 [Corporate Presentation for use at the 38th Annual J.P. Morgan Healthcare Conference on January 14, 2020](#)  
Cover Page Interactive Data File (embedded within the Inline XBRL document)

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**Signature**

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Date: January 13, 2020

ALEXION PHARMACEUTICALS, INC.

By: /s/ Doug Barry

Name: Doug Barry

Title: Vice President, Corporate Law

The Alexion logo features the word "ALEXION" in a bold, sans-serif font. A thin red line arches over the letters "E" and "X", ending in a small red arrowhead pointing to the right.

38th Annual J.P.  
Morgan Healthcare  
Conference

Ludwig Hantson, Ph.D.  
Chief Executive Officer

JANUARY 14, 2020

A photograph of two women smiling. On the left is an older woman with short, wavy white hair, wearing a blue top. On the right is a younger woman with shoulder-length blonde hair, wearing a bright yellow top and a colorful beaded necklace. They are both looking towards the camera with warm expressions.

Katie with her mom Living with PNH

This presentation contains forward-looking statements, including statements related to: guidance regarding anticipated financial results for 2019 (and all of the assumptions and estimates related to such guidance); the strength of our business and continued growth; plans to expand the Company's pipeline; Company's goal of continuing to build on momentum as the year progresses; future plans for, and the timing for, the commencement of future clinical trials and the expected timing of the receipt of results of certain clinical trials and studies, including clinical programs for ULTOMIRIS in aHUS, NMOSD, HSCIT-TMA, ALS, PPMS and a subcutaneous administration in PNH and aHUS and for ALXN1830 in WAIHA and gMG; potential benefits of current products and products under development and in clinical trials; plans for development programs with third parties including, Eidos, Affibody, Dicerna, Zealand, Stealth and Complement Pharma; the potential to treat a broad range of complement mediated diseases with the product to be developed with Zealand; the anticipated closing of the Achillion acquisition; and Alexion's future clinical, regulatory, and commercial plans for ULTOMIRIS and other products and product candidates. 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 principal product (SOLIRIS); our ability to facilitate the timely conversion from SOLIRIS to ULTOMIRIS; payer, physician and patient acceptance of ULTOMIRIS as an alternative to SOLIRIS; appropriate pricing for ULTOMIRIS; future competition from biosimilars and novel products; 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 failure 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 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 planned 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; 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 ULTOMIRIS brought by third parties against Alexion and inter partes review petitions submitted by third parties); 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; the possibility that expected tax benefits will not be realized; 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, including investigations of Alexion by the U.S. Securities and Exchange Commission (SEC) and U.S. Department of Justice; 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, 2019 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 presentation 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. The non-GAAP results 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 collaborations, acquired in-process research and development assets, impairment of intangible assets, change in value of strategic equity investments, litigation charges, gain or loss on sale of a business or asset 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 2019 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 nine month periods ended September 30, 2019 and 2018 and projected twelve months ending December 31, 2019.

Amounts may not foot due to rounding.



# OUR MISSION

Serving patients and their families is our unwavering mission – they are our guiding star and they inspire us to continue to find answers.

We act with integrity, urgency, and discipline because we know that lives are at stake.

**>7,000**  
rare diseases identified

**Only 500**  
rare diseases have approved therapies

**~30M**  
patients diagnosed in US, 50% are children

Albie Living with LAL-D





**4**  
Transformative  
Medicines

Treating  
**6**  
Rare Diseases

**19**  
Clinical-stage  
development  
programs  
planned for  
2020

Ambition for  
**10**  
launches by  
2023

**Redefining  
Rare  
Neurology**

Ambition to treat  
**4x**  
U.S. Neurology  
patients  
by **2025**



Ambition for  
continued  
**Double-digit  
Revenue  
Growth**

# FOUR TRANSFORMATIVE MEDICINES ACROSS SIX RARE DISEASES



## ULTOMIRIS®

(RAVULIZUMAB-CWVZ)  
FOR

PNH  
aHUS

## SOLIRIS®

(ECULIZUMAB)  
FOR

PNH  
aHUS  
gMG  
NMOSD

## STRENSIQ®

(ASFOTASE ALFA)  
FOR

HPP

## KANUMA®

(SEBELIPASE ALFA)  
FOR

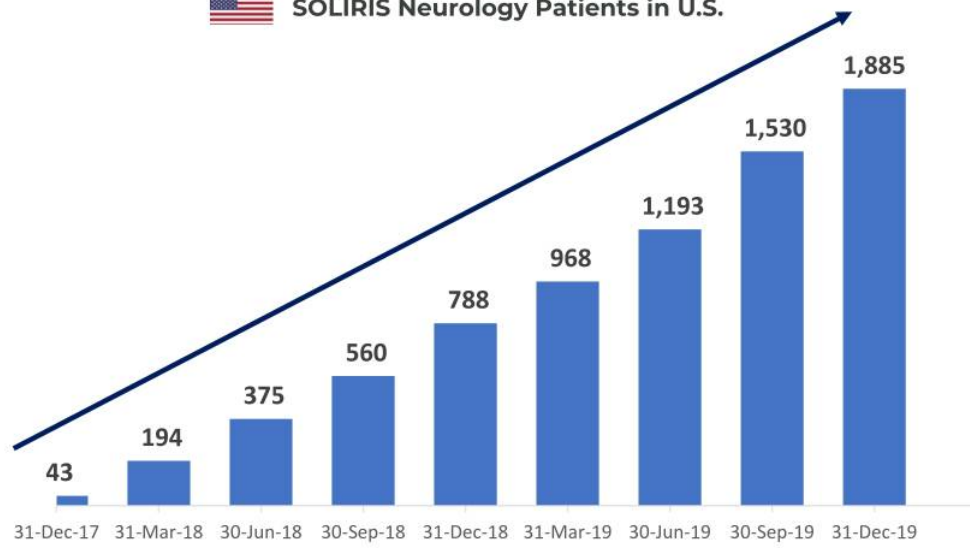
LAL-D

# TRANSFORMED OUR DEVELOPMENT PORTFOLIO: 19 DEVELOPMENT PROGRAMS PLANNED ACROSS 10 ASSETS



# 2025 AMBITION: 4X EXPANSION OF TREATED U.S. NEUROLOGY PATIENTS WITH SOLIRIS AND ULTOMIRIS

 SOLIRIS Neurology Patients in U.S.



***In Less Than 2 Years, Neurology has Grown to be Largest Franchise by Patient Volume***

# STRONG FINANCIAL EXECUTION WITH AMBITION TO MAINTAIN DOUBLE-DIGIT REVENUE GROWTH



**Achieved >20% Top-line Revenue Growth FY2019 vs. FY2018\***

\*Top-line revenue growth is based on preliminary financial results for the year ended December 31, 2019 and is subject to further review and adjustment

# Clear Strategy for Value Creation 2020 and Beyond



AIRA  
LIVING WITH HPP



VICTOR  
LIVING WITH PNH



TRENDY BROTHERS  
LIVING WITH LAL-D



MONIKA  
LIVING WITH gMC

LEAD

- ✓ **ULTOMIRIS is the market leader in PNH\***
- ✓ **Continued facilitated conversion to ULTOMIRIS in aHUS**

EXPAND

- ✓ **Expanding the C5 Franchise beyond base business**
- ✓ **Neurology is largest franchise by patient volume in U.S.**

DIVERSIFY

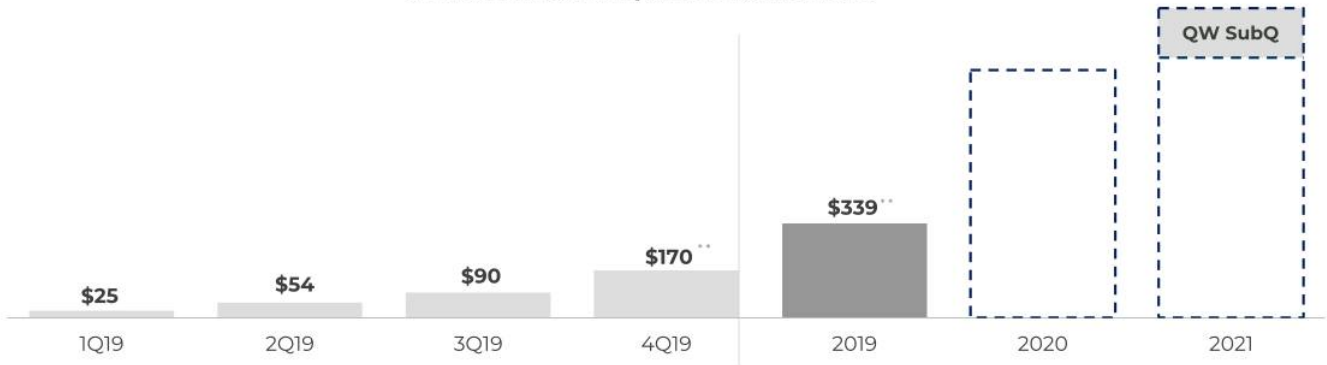
- ✓ **Strong FCF and low leverage ratio provide BD capacity**
- ✓ **Additional opportunities with internal R&D efforts**

\*In previously characterized top three geographies (US, Germany, Japan)

ULTOMIRIS IS THE  
MARKET LEADER



ULTOMIRIS PNH/aHUS Revenues



Illustrative; Not To Scale

Reported PNH Conversion

	Jan 31, 2019	Feb 27, 2019	Mar 19, 2019	Apr 24, 2019	Jul 22, 2019	Oct 21, 2019	Nov 19, 2019	Dec 3, 2019	Jan 10, 2020
	3%	8%	14%	22%	40%	51%	-	56%	59%
	-	-	-	-	-	45%	50%	56%	60%
	-	-	-	-	-	15%	28%	35%	44%

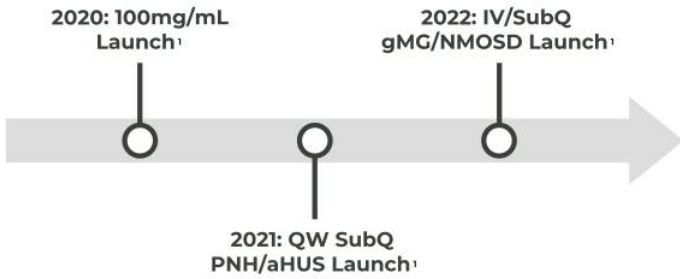


- **Largest PNH studies representative of real-world patient population**
- **Unique value proposition supported by patient preference for Q8W infusion**

**Similar Best-in-class aHUS Conversion Ambition; EU launch anticipated 1H2020**

\*In previously characterized top three geographies (US, Germany, Japan)

\*\*ULTOMIRIS revenues based on preliminary financial results for the year ended December 31, 2019 and is subject to further review and adjustment.



### ULTOMIRIS: 100mg/mL High Concentration

- Innovation aligned with patient preference for shorter infusion time
- Reduces infusion time by >50% to ~45 minutes
- Potential 2020 approval and launch

### ULTOMIRIS: Once-weekly Subcutaneous

- Commercially validated, patient-friendly device
- Once-weekly infusion provides consistency for patients
- Phase 3 core data read-out 2Q2020; Potential 2021 Launch in PNH/aHUS
- Pursuing SubQ bridging strategy in gMG and NMOSD

Provides self-administration option for subset of patients indicating preference for SubQ administration



Low volume (7mL)

On-body device

10 minute infusion

West SMARTDOSE

Patients preferring SubQ administration seek **control** over disease, **comfort** in reliability of treatment regime and **confidence** in treatment's consistent efficacy and safety

<sup>1</sup>Subject to approval of regulatory agencies

# EXPANDING OUR C5 FRANCHISE

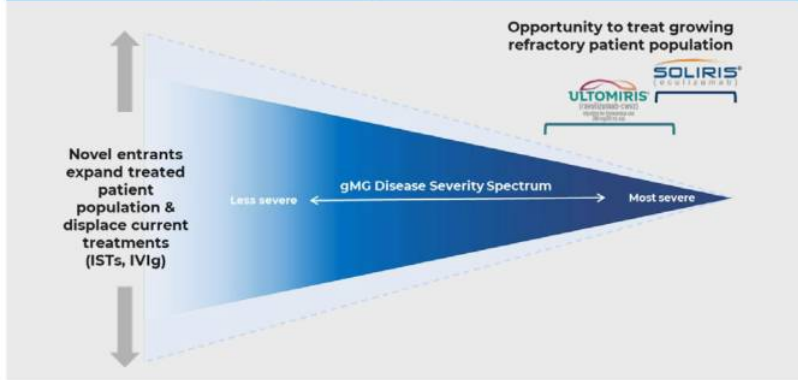
# TRANSFORMED OUR DEVELOPMENT PORTFOLIO: EXPANDING OUR C5 FRANCHISE



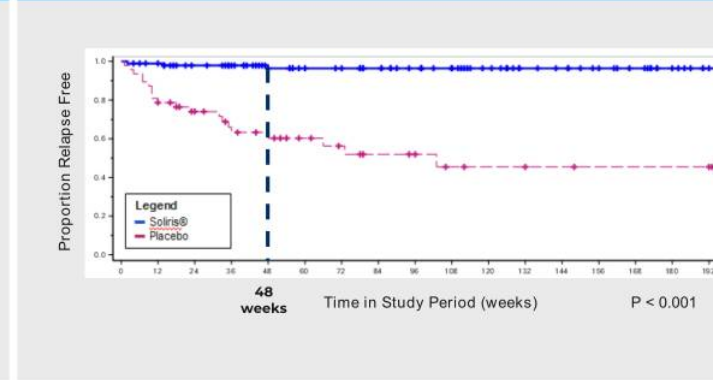
1. Structured as an option to acquire  
 2. Subject to transaction closure  
 3. Exclusive license to develop and commercialize in Japan

# STRONG PRODUCT PROFILE WITH SIGNIFICANT MARKET EXPANSION OPPORTUNITY

## ULTOMIRIS and FcRn Assets Further Expand gMG Target Population



## NMOSD Phase 3 PREVENT study results shows 98% patients relapse free at 48 weeks



Ambition to treat 4x Neurology Patients with SOLIRIS and ULTOMIRIS by 2025 in the U.S.

# EXPANDING BEYOND PNH/AHUS WITH ULTOMIRIS AND INNOVATIVE C5 PROGRAMS



Current Portfolio Focus		Future Growth Opportunities	
<b>gMG</b>	<ul style="list-style-type: none"> <li>SOLIRIS gMG fastest growing indication in Q3</li> <li>ULTOMIRIS Ph3 gMG program underway; similar best-in-class conversion ambition</li> </ul>	<b>ALS</b>	<ul style="list-style-type: none"> <li>Preclinical animal models, clinical biomarker data support terminal complement involvement</li> <li>15-20,000 patients in US, EU5, Japan</li> <li>Ph3 study planned for 2020</li> </ul>
<b>NMOSD</b>	<ul style="list-style-type: none"> <li>SOLIRIS first therapy approved in NMOSD</li> <li>Japan approval in November 2019; launch underway</li> <li>U.S. and Germany launches underway</li> </ul>	<b>HSCT-TMA</b>	<ul style="list-style-type: none"> <li>Clinical evidence for effectiveness of SOLIRIS HSCT-TMA</li> <li>Estimated ~5,000 addressable patients</li> </ul>
		<b>NEW</b> <b>CM-TMA</b>	<ul style="list-style-type: none"> <li>Clinical evidence for effectiveness of SOLIRIS CM-TMA</li> <li>Estimated ~2,000 addressable patients</li> </ul>
		<b>PPMS</b>	<ul style="list-style-type: none"> <li>Elevated levels of C3 and C4 in PPMS at time disease progression</li> </ul>

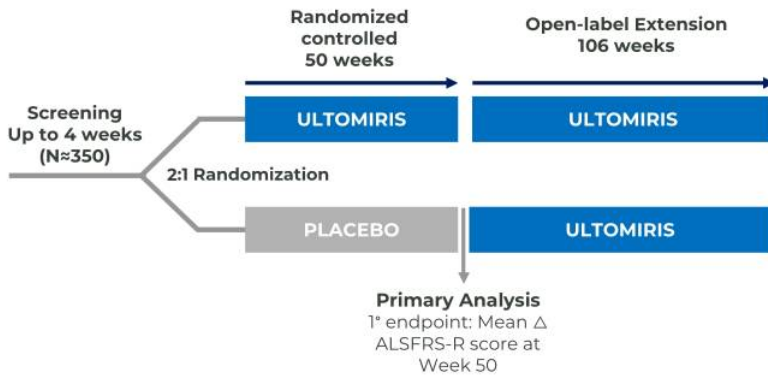
## Next Gen C5 Assets Provide Growth Opportunities in Neurology and Other Rare Diseases

<b>ALXN1720</b> <i>Novel bi-specific developed in-house</i>	<b>NEW – Renal Basket Study</b>	<b>ALXN1810</b> <i>Novel formulation leveraging Halozyme PH20 technology</i>
----------------------------------------------------------------	---------------------------------	---------------------------------------------------------------------------------

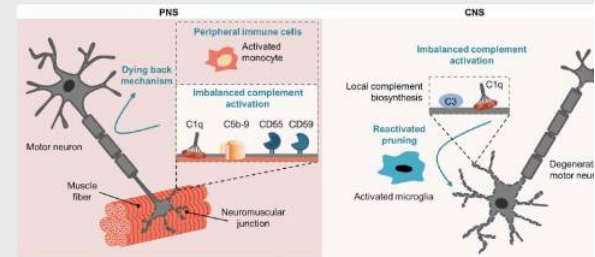
# ALS IS A HIGH-RISK, HIGH-REWARD PROGRAM WITH COMPELLING SCIENTIFIC RATIONALE

## ULTOMIRIS ALS Phase 3 Trial Design

Plan to initiate Ph3 Study 1Q2020



## Role of Complement in ALS

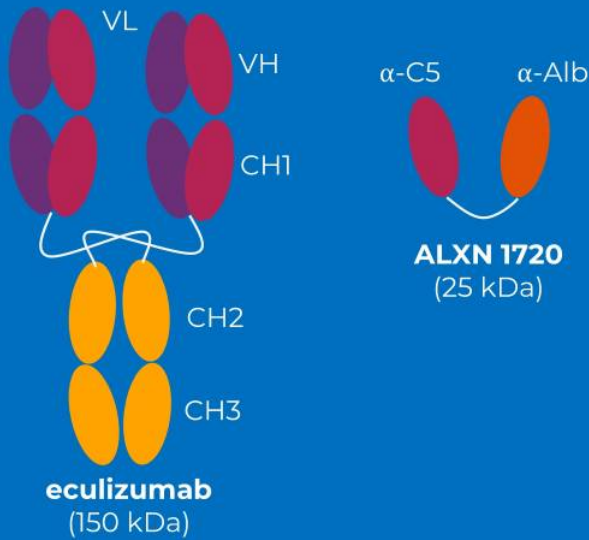


Source: Kjeldgaard, A.-L., Molecular Immunology (2018), <https://doi.org/10.1016/j.molimm.2018.06.001>

Inappropriate activation of complement system plays a role in the pathogenesis of ALS

- ALS is a neurodegenerative disease characterized by motor neuron degeneration leading to progressive muscle weakness
- Scientific rationale supports potential role of complement, including **MAC deposition in ALS**
  - Elevated complement activation products in ALS serum and CSF
- Estimated **15-20K** addressable population in US, EU and Japan

## ALXN1720: Tailor-Made for SC at Only 25 kDa



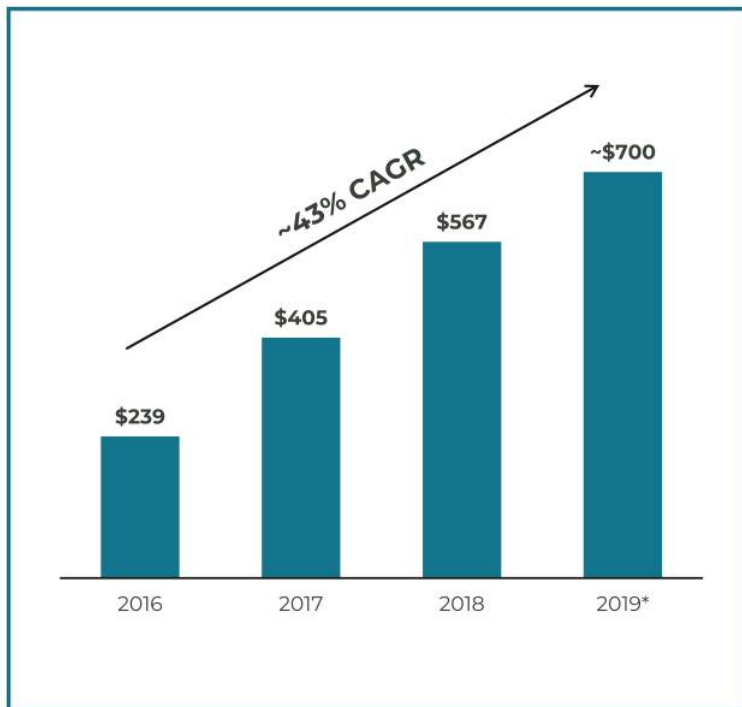
## ALXN1720 Continues Complement Leadership

- Bi-specific mini-body that binds and prevents activation of human C5
- Specifically designing for long-acting, small volume SubQ dosing:
  - **25 kDa size** (compared to ~150 kDa for most antibodies) with potential for auto-injector or pre-filled syringe
  - **Long half-life by binding to human serum albumin**
- **Ongoing Phase 1 SAD/MAD study (N=72)**
- Ability to **switch between current C5 therapies** without immunogenicity issues and **combine with FcRn therapies** without reduction of half-life
- Strategic indication selection ongoing for Ph2 trial; **Plan to initiate PoC study 1H2021**
- **Ideally suited for new larger-population rare indications**



# DIVERSIFYING BEYOND C5, EXPANDING OUR ADDRESSABLE PATIENT POPULATIONS

# METABOLIC PORTFOLIO CONTINUES CONSISTENT GROWTH TRAJECTORY



## STRENSIQ for Hypophosphatasia (HPP)

- Launched and Reimbursed in 7 countries
- Improving patient diagnosis with Caliper Initiative



## KANUMA for Lysosomal Acid Lipase Deficiency (LAL-D)

- Improving funding agreements and securing access
- Launched and Reimbursed in 9 countries

\* Revenues based on preliminary financial results for the year ended December 31, 2019 and is subject to further review and adjustment.

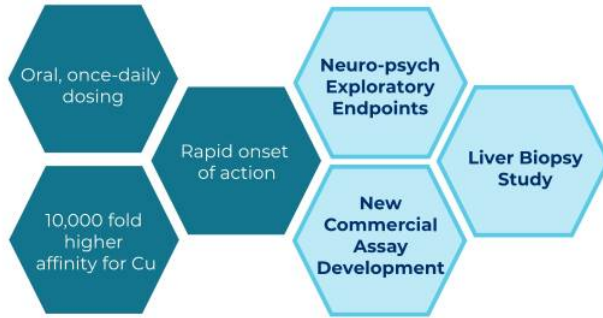
# TRANSFORMED OUR DEVELOPMENT PORTFOLIO: DIVERSIFYING BEYOND C5



■ Hematology   
 ■ Nephrology   
 ■ Metabolics   
 ■ Neurology   
 ■ Cardiology   
 ■ Other/TBD

# ALXN1840: OPPORTUNITY TO TRANSFORM STANDARD OF CARE IN WILSON DISEASE

## Building Blocks for Strong Label at Launch



## ALXN1840 Blockbuster Potential

- Wilson disease is rare disorder characterized by copper accumulation in the liver, brain, and other organs
- Risk of cirrhosis or liver failure; other symptoms include fatigue, pain, swelling, confusion, psychosis and psychiatric disorders
- Need unaddressed by current chelators because of poor compliance and potential for neurological worsening
- **US and EU 10,000+ addressable patient population**
- **New development efforts improve diagnosis and strength label at launch:**
  - **Neuro-psych endpoints, Liver Biopsy Study, Commercial Assay**



**2018**

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



**1Q2020**

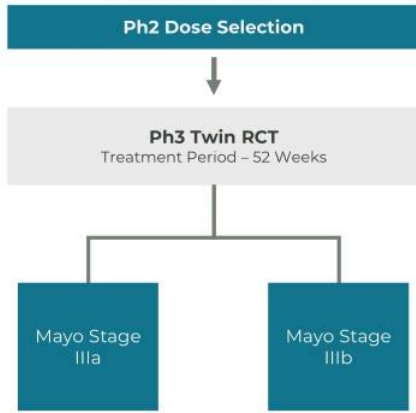
Ph3 FoCuS Trial Enrollment Completion



**1H2021**

Potential Top Line Results

**CAEL-101 Phase 2/3 Program Design**



*Primary Endpoint: Overall Survival*  
*Secondary Endpoints: Patient Function, QoL, and Cardiac Imaging*

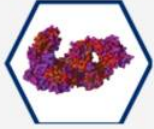
- AL Amyloidosis caused by deposition of circulating misfolded immunoglobulin light chains resulting in severe organ damage
- **Median survival <18 months of diagnosis**
- **Affects >30,000 patients in the US and EU5**

- **Chemotherapeutic SoC – none yet approved – does not address underlying plaque deposits in organs**
- Heart and kidney most frequently affected

- **Proof of Concept in Phase 1a/b study** suggests potential for improved overall survival (93% at 18.6 months)

**Alexion maintains option to acquire post-Ph3 data**

**ALXN1830**



- Proof of concept established in Ph1b/2a
- High specificity to IgG
- No reduction in albumin observed
- Rapid onset of action

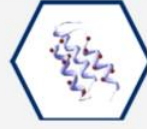
**Generalized Myasthenia Gravis (gMG)**

- Moving forward with SubQ formulation
- Targeted mild-to-moderate patients; majority of total 60-80,000 gMG patient population

**Warm Autoimmune Hemolytic Anemia (WAIHA)**

- Estimated 65K Patients in U.S.
- No approved treatment options

**ABY-039**



- High affinity protein ligand
- Extended half-life due to albumin-binding domain
- Only 19kDa; optimized for SubQ dosing

**Numerous Indication Opportunities in IgG-mediated Autoimmune Rare Diseases with Burdensome, Chronic SoC**

WAIHA	1H 2020	2H 2020
ALXN1830 IV	Ph2	
<b>gMG</b>		
ALXN1830 SC	SAD/MAD	Ph2

SAD/MAD	1H 2020	2H 2020
ABY-039	SAD/MAD	

## Top-Line Phase 2 Data Showed Meaningful Improvement in Hgb, Transfusion needs, FACIT-Fatigue

	Baseline N=11 Mean (SD)	Week 24 N=11 Mean (SD)
<b>Lab Parameters</b>		
Hgb (g/dL)	7.9 (1.42)	10.3 (1.66)
LDH (xULN)	1.06 (0.321)	1.04 (0.181)
Reticulocytes (10 <sup>9</sup> /μl)	219 (78.1)	135 (66.3)
Total bilirubin (mg/dL)	2.17 (1.118)	1.35 (0.798)
Direct bilirubin (mg/dL)	0.51 (0.220)	0.37 (0.207)
PNH red cell clone size (%)	54 (24.7)	84 (22.1)*
C3 fragment deposition on PNH RBCs (%)	30 (24.7)	8 (9.8)**
<b>Quality of Life</b>		
FACIT-Fatigue	34 (14.1)	45 (8.2)

\* N=7; for four patients, samples were out of stability range.  
 \*\* N=8; pending results for 3 patients.  
 \*\*\* Scores based on the Functional Assessment of Chronic Illness Therapy Fatigue (FACIT) Fatigue Scale V4. Score range 0-52. A score of less than 30 indicates severe fatigue.

Source: Achillion

- Transfusion needs dramatically reduced with one patient receiving one transfusion during the trial, as compared to 34 transfusions (58 units) in 10 patients in the 6 months prior to screening

**Potential Danicopan + C5 Inhibitor Combination: Convenient daily oral treatment for minority of PNH patients experiencing EVH**

## Factor D Platform with two clinical stage assets

Danicopan (ACH-4471)	ACH-5228
Oral TID Dosing	Oral BID Dosing
Ongoing Phase 2 in C3G	Potential best-in-class oral Factor D inhibitor
Planned Phase 3 in PNH with EVH	

## Factor D is a critical control point for complement system's alternative pathway (AP)

Opportunity to treat range of rare diseases



Note: Achillion acquisition subject to customary closing conditions, including receipt of approval from anti-trust authorities

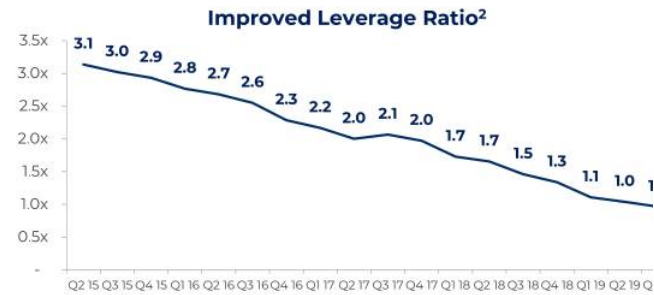
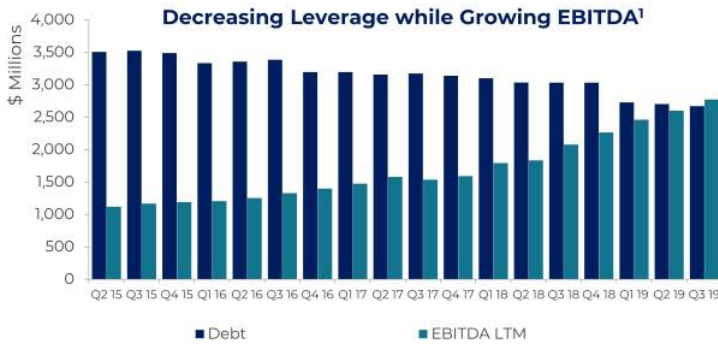
# DISCIPLINED BUSINESS DEVELOPMENT PROVIDES ADDITIONAL PORTFOLIO GROWTH OPPORTUNITIES

## Optimize Deal Structure to Minimize Risk

**Preclinical/Discovery Collaborations:** Low Upfront, Back-end weighted dependent on program success

**Clinical-stage Collaborations or Acquisitions:** Larger Upfront due to established Proof of Concept, Smaller royalties/milestones

## Significant Capacity Remains For Future BD



<sup>1</sup>EBITDA is a non-GAAP financial measure. Reconciliation of EBITDA to the most comparable GAAP measure provided in the company's financial results for each quarter at [www.ir.Alexion.com/events-and-presentations](http://www.ir.Alexion.com/events-and-presentations)  
<sup>2</sup>Leverage ratio is defined as Net Debt/ULTM EBITDA. Net debt is defined as total debt less cash.



# Continuing Momentum into 2020

# VALUE CREATION STRATEGY FOCUSED ON LEADING IN PNH/AHUS, EXPANDING & DIVERSIFYING OUR BUSINESS BEYOND C5

### LEAD

**SOLIRIS<sup>®</sup>**  
(eculizumab)  
Approved by FDA/EMA/EMA

**ULTOMIRIS<sup>™</sup>**  
(ravulizumab-cwvz)  
Approved for intravenous use

**C5 Franchise (PNH & aHUS)**

### EXPAND

<b>ULTOMIRIS</b>	<ul style="list-style-type: none"> <li>• gMG</li> <li>• NMOSD</li> <li>• ALS</li> <li>• HSCT-TMA</li> <li>• PPMS</li> <li>• Others to be announced</li> </ul>
<b>Next Generation C5 Programs</b>	<ul style="list-style-type: none"> <li>• ALXN1720</li> <li>• ALXN1810</li> <li>• Weekly SC</li> </ul>

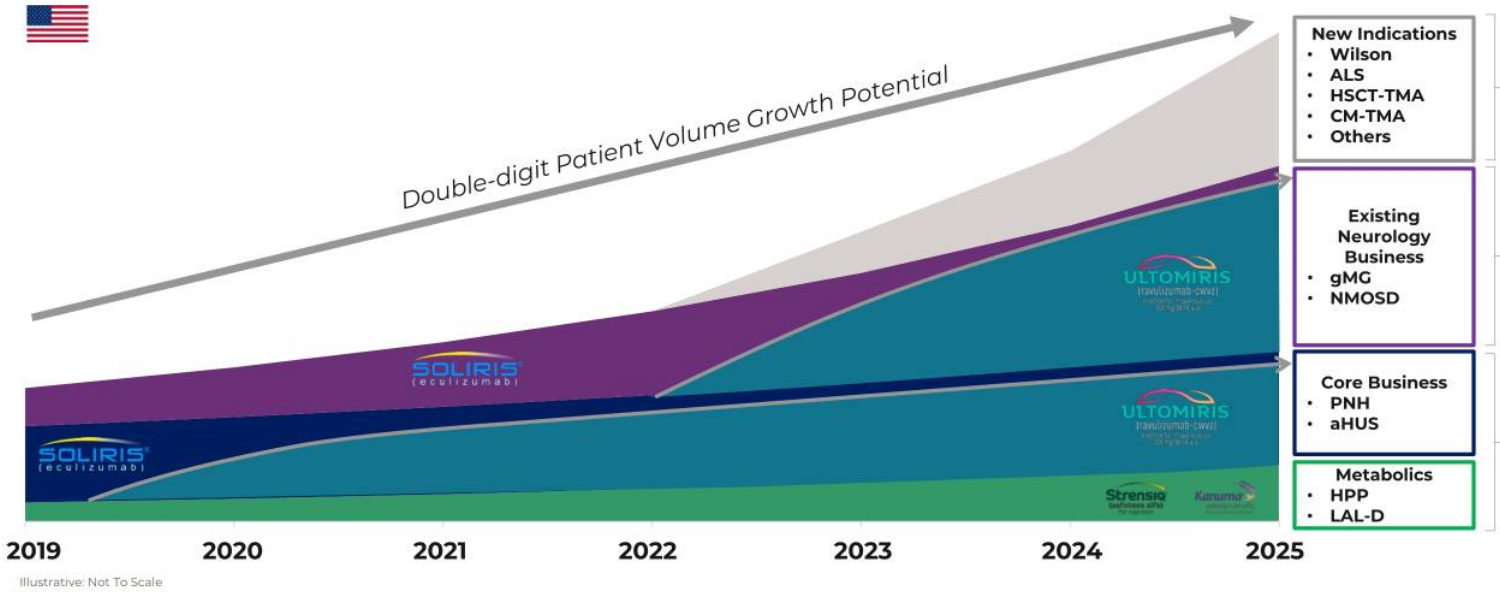
### DIVERSIFY

<b>STRENSIQ<sup>®</sup></b>	• HPP
<b>KANUMA<sup>®</sup></b>	• LAL-D
<b>ALXN1840</b>	• Wilson Disease
<b>FcRn</b> <small>ALXN1830, ABY-039</small>	• WAIHA, gMG • Rare autoimmune
<b>CAEL-101<sup>1</sup></b>	• AL Amyloidosis
<b>Eidos: AG10<sup>3</sup></b>	• ATTR
<b>Achillion<sup>2</sup>: Factor D</b>	• C3G • EVH in PNH • Other complement
<b>Further Disciplined BD</b>	

**Ambition to maintain double-digit revenue Growth and a focus on strategic capital deployment**

1. Structured as an option to acquire  
 2. Subject to transaction closure  
 3. Exclusive license to develop and commercialize in Japan

# CLEAR PATH TO VOLUME GROWTH IN CURRENT PORTFOLIO



**Conversion Drives Long-term Sustainability in Base Business; Additional Growth in Neurology and New Indication Opportunities**

**1 TRANSFORMED R&D PORTFOLIO**

- 19 development programs across 10 assets
- Disclosed plans for two new programs: ALXN1810 renal basket study and ULTOMIRIS CM-TMA

**2 RARE DISEASE COMMERCIAL EXCELLENCE**

- ULTOMIRIS is market leader in PNH (>50% Conversion in US, Germany)
- Ambition to make ULTOMIRIS market leader in aHUS
- 2025 Ambition: 4x expansion of gMG and NMOSD treated patient population in U.S.

**3 STRONG FINANCIAL EXECUTION**

- >20% top-line revenue growth FY2019 vs. FY2018<sup>1</sup>
- Focused on strategic capital deployment:
  - Optimized leverage ratio (~1.0x Net Debt/LTM EBITDA)<sup>2,3</sup>

**4 CLEAR STRATEGY FOR VALUE CREATION**

- **LEAD:** Establish ULTOMIRIS as market leader in PNH and aHUS
- **EXPAND:** Continued innovation in C5
- **DIVERSIFY:** Looking beyond C5 to expand our leadership in rare disease

<sup>1</sup>Top-line revenue growth is based on preliminary financial results for the year ended December 31, 2019 and is subject to further review and adjustment.  
<sup>2</sup>EBITDA is a non-GAAP financial measure. Reconciliation of EBITDA to the most comparable GAAP measure provided in the company's financial results for each quarter at [www.ir.Alexion.com/events-and-presentations](http://www.ir.Alexion.com/events-and-presentations)  
<sup>3</sup>Leverage ratio is defined as Net Debt/LTM EBITDA. Net debt is defined as total debt less cash.



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LIVING WITH HPP



JUSTICE  
LIVING WITH aHUS



LIVING W

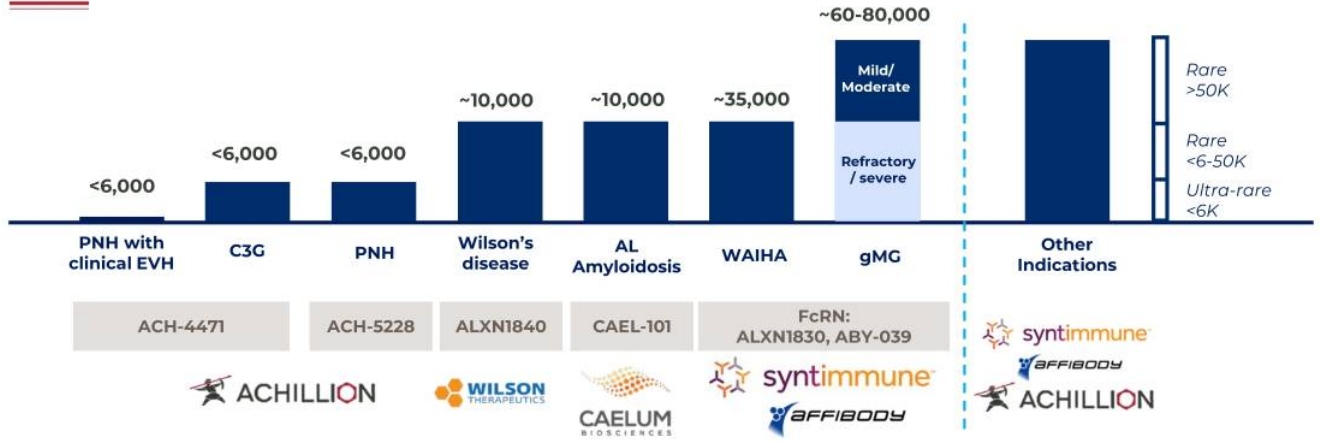


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# APPENDIX

# FOCUS ON RARE DISEASE ASSETS TO EXPAND ADDRESSABLE PATIENT POPULATION



The logo for ALEXION is centered on a dark blue background. It features the word "ALEXION" in a white, bold, sans-serif font. A white swoosh underline starts under the 'A' and extends to the right, ending with a small red triangle pointing downwards. The swoosh is positioned above the letters 'L', 'E', 'X', and 'I', and slightly below the 'O' and 'N'.

**ALEXION**

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