



**DAVID BRENNAN**  
**INTERIM CEO**

35th Annual  
J.P. Morgan Healthcare Conference  
January 9, 2017



# Forward-Looking Statements

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This presentation contains forward-looking statements, including statements related to anticipated financial results for 2016, assessment of Alexion's financial position and commercialization efforts, medical benefits and commercial potential for Soliris®, Strensiq® and Kanuma®, medical and commercial potential of each of Alexion's product candidates, launch expectations for Strensiq and Kanuma, and plans for regulatory filings and clinical programs for each of our product candidates. Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ from those expected, including for example, decisions of regulatory authorities regarding marketing approval or material limitations on the marketing of our products, delays, interruptions or failures in the manufacture and supply of our products and our product candidates, progress in establishing and developing commercial infrastructure, failure to satisfactorily address matters raised by the FDA and other regulatory agencies, the possibility that results of clinical trials are not predictive of safety and efficacy of our products in broader patient populations in the disease studied or other diseases, the risk that strategic transactions will not result in short-term or long-term benefits, the possibility that current results of commercialization are not predictive of future rates of adoption of Soliris in PNH, aHUS or other diseases, the possibility that clinical trials of our product candidates could be delayed or that additional research and testing is required by regulatory agencies, including for ALXN1210, the adequacy of our pharmacovigilance and drug safety reporting processes, the risk that third party payers (including governmental agencies) will not reimburse or continue to reimburse for the use of our products at acceptable rates or at all, risks regarding government investigations, including investigations of Alexion by the SEC and DOJ, the risk that regulatory filings are delayed, the risk that estimates regarding the number of patients with PNH, aHUS, HPP, or LAL-D are inaccurate, and a variety of other risks set forth from time to time in Alexion's filings with the U.S. Securities and Exchange Commission, including but not limited to the risks discussed in Alexion's Quarterly Report on Form 10-Q for the period ended September 30, 2016 and in our other filings with the U.S. Securities and Exchange Commission. Alexion does not intend 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 U.S. GAAP, this presentation also contains non-GAAP financial measures that we believe, when considered together with the GAAP information, provide investors and management with supplemental information relating to operating performance, trends and prospects that promote a more complete understanding of Alexion's operating results and financial position during different periods. The non-GAAP results exclude the impact of the following GAAP items: share-based compensation expense, fair value adjustment of inventory acquired, amortization of purchased intangible assets, changes in fair value of contingent consideration, acquisition-related costs, restructuring expenses, upfront and milestone payments related to licenses and collaborations and 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. We typically exclude certain GAAP items that management does not believe affect our basic operations and that do not meet the GAAP definition of unusual or non-recurring items. Other companies may define these measures in different ways. Further information relevant to the interpretation of adjusted financial measures, and reconciliations of these adjusted financial measures to the most comparable measures, may be found in our press releases issued January 4, 2017 and October 27, 2016.

O u r M i s s i o n :

Alexion discovers,  
develops, and  
delivers innovative  
therapies for  
patients with  
devastating and rare  
diseases



# Alexion Today



**3**

**DRUGS**

Approved for  
devastating,  
ultra-rare diseases



**4**

**ULTRA-RARE  
DISEASES**

Breakthrough  
innovations



**10**

**PROGRAMS**

In clinical  
development



**50**

**COUNTRIES**

Serving  
patients



**3000**

**TALENTED  
COLLEAGUES**

Worldwide



**>3B**

**REVENUE\***

In 2016

# Corporate Highlights

## 3Q16 Quarterly Performance

**\$799M**

Total Revenue

**20%** YoY  
Revenue Growth

**23%** YoY  
Volume Growth

**\$0.42**  
GAAP EPS

**\$1.23**  
Non-GAAP EPS

## R&D Updates

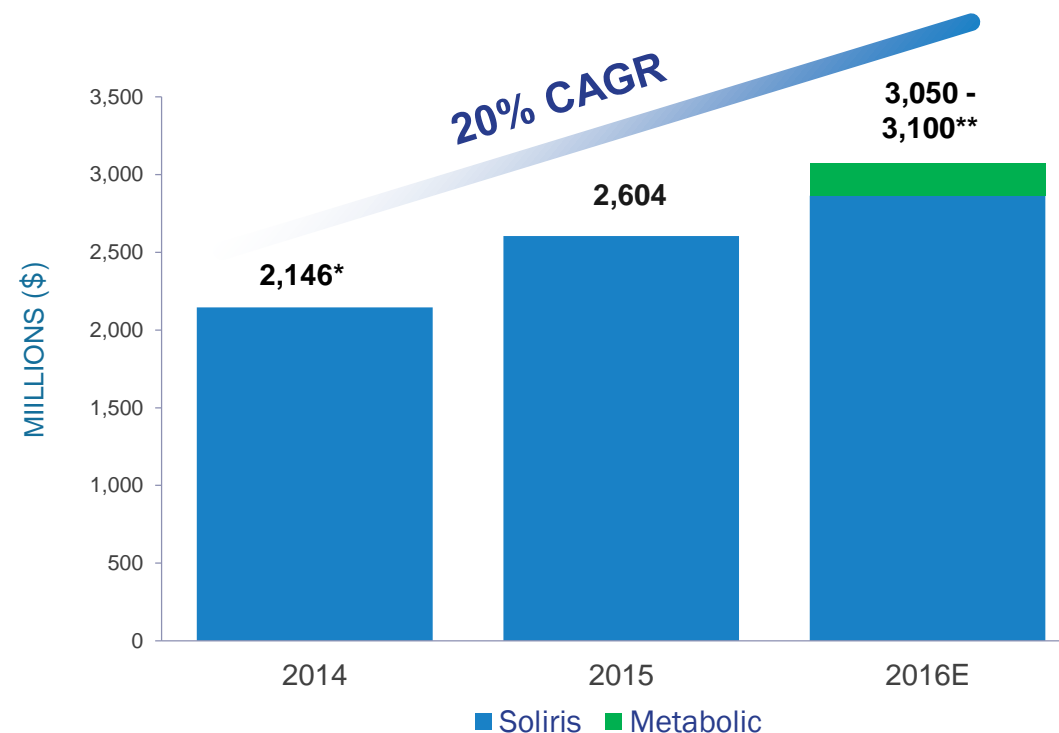
- **Soliris® in Refractory gMG**
  - Regulatory submissions filed in the US and Europe
- **ALXN1210 in PNH (Q8 Week Dosing)**
  - Dosing underway in multinational registration trial
  - ODD granted in US and Europe
- **ALXN1210 in aHUS (Q8 Week Dosing)**
  - Recruitment underway in multinational registration trial
- **ALXN1210 Subcutaneous**
  - Enrollment complete in Phase 1 healthy volunteer study
- **Eculizumab in NMOSD**
  - Multinational registration trial progressing
- **SBC-103 in MPS IIIB**
  - Phase 1/2 study progressing
- **Samalizumab in Solid Tumors and AML**
  - Dosing underway with innovative immunomodulatory antibody targeting CD200

# Financial Outlook

## 2016 Financial Guidance

\$ Millions, Except EPS	GAAP Guidance <sup>(1)</sup>	Non-GAAP Guidance <sup>(1)</sup>
<b>Total Revenue</b>	<b>\$3,050 to \$3,100</b>	<b>\$3,050 to \$3,100</b>
<b>Earnings Per Share</b>	<b>\$1.79 to \$2.09</b>	<b>\$4.50 to \$4.65</b>

## Total Revenue



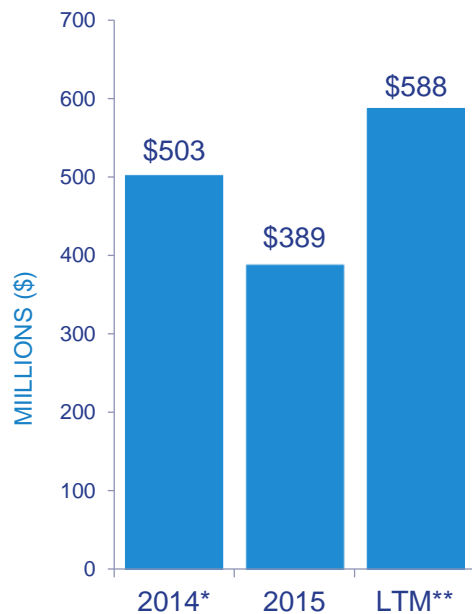
\*2,146M excludes ~\$88M in prior years shipments; \*\*2016 Total Revenue Guidance of \$3,050M to \$3,100M; Guidance most recently provided January 4, 2017.

<sup>(1)</sup> A reconciliation of our GAAP to non-GAAP financial results is set forth in our third quarter 2016 financial results press release issued October 27, 2016.

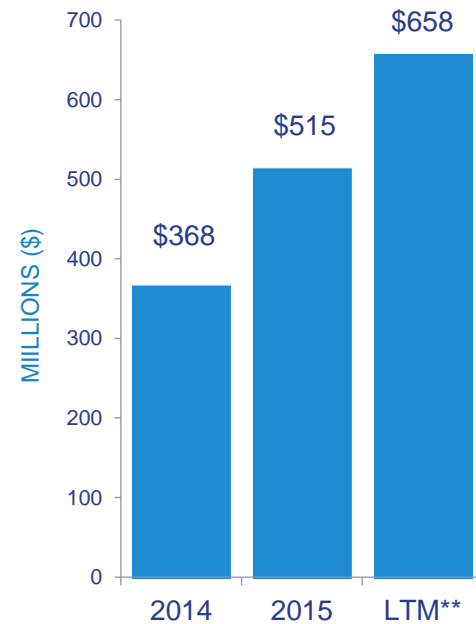
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# Capital Allocation Strategy

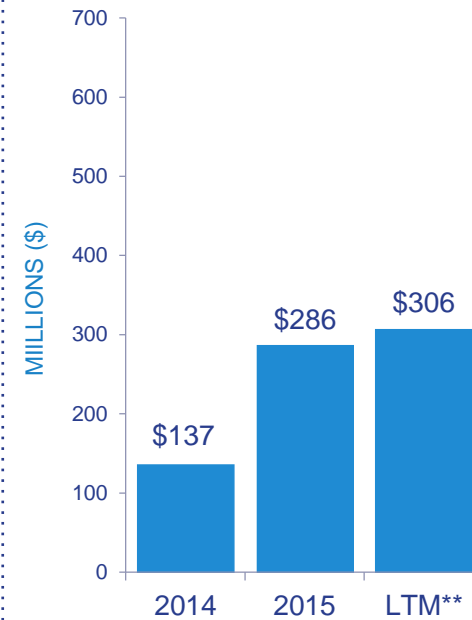
Free Cash Flow<sup>(1)</sup>



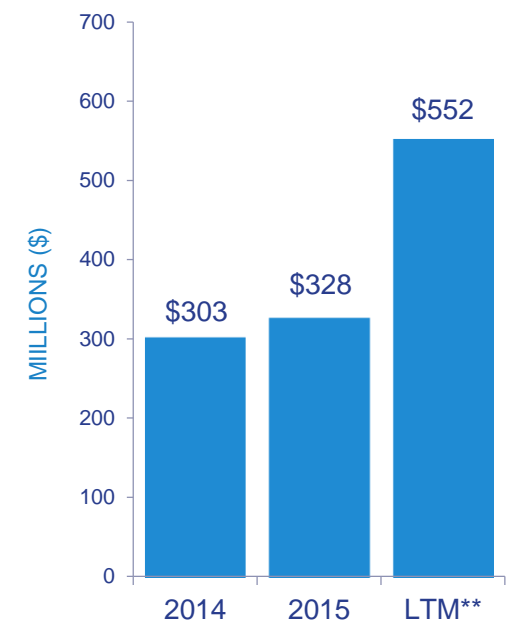
Non-GAAP R&D Investment



Capital Expense



Share Repurchase



<sup>(1)</sup>Free Cash Flow = Cash Flow from Operations less Capital Expenditures

\*2014 Revenues include \$88M in prior years shipments

\*\*LTM = Last 12 months, Q4 2015 through Q3 2016

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# Advancing Leadership Position in Complement

1

## Leverage Unique Rare Disease Infrastructure

- Continued growth ahead in PNH and aHUS
- Commercial, clinical operations, and regulatory infrastructure to support future growth

2

## Expand to New Indications

- **Soliris gMG**: Regulatory submissions filed in the US and EU
- **Eculizumab NMOSD**: Registration trial progressing

3

## Strengthen Patent Position

- Soliris CoM patent in the U.S. and key EU countries through 2021/2020
- Continue to expand and strengthen global Soliris IP portfolio
- ALXN1210 CoM patent in the U.S. and EU through 2035

4

## Drive Continued Innovation

- ALXN1210 Phase 3 studies underway in PNH and aHUS
- ALXN1210 SC Phase 1 enrollment complete
- 4 additional complement inhibitors in development



# Building a Leading Metabolic Franchise



**2**

**MARKETED  
PRODUCTS**

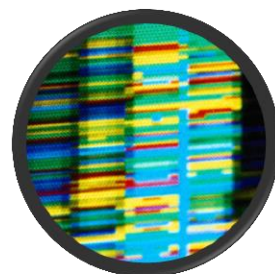
Approved for  
devastating,  
ultra-rare diseases



**2**

**RARE  
DISEASES**

Breakthrough  
innovations



**1**

**PROPRIETARY  
PLATFORM**

Protein expression for  
innovative ERT's



**2**

**PROGRAMS**

In clinical  
development

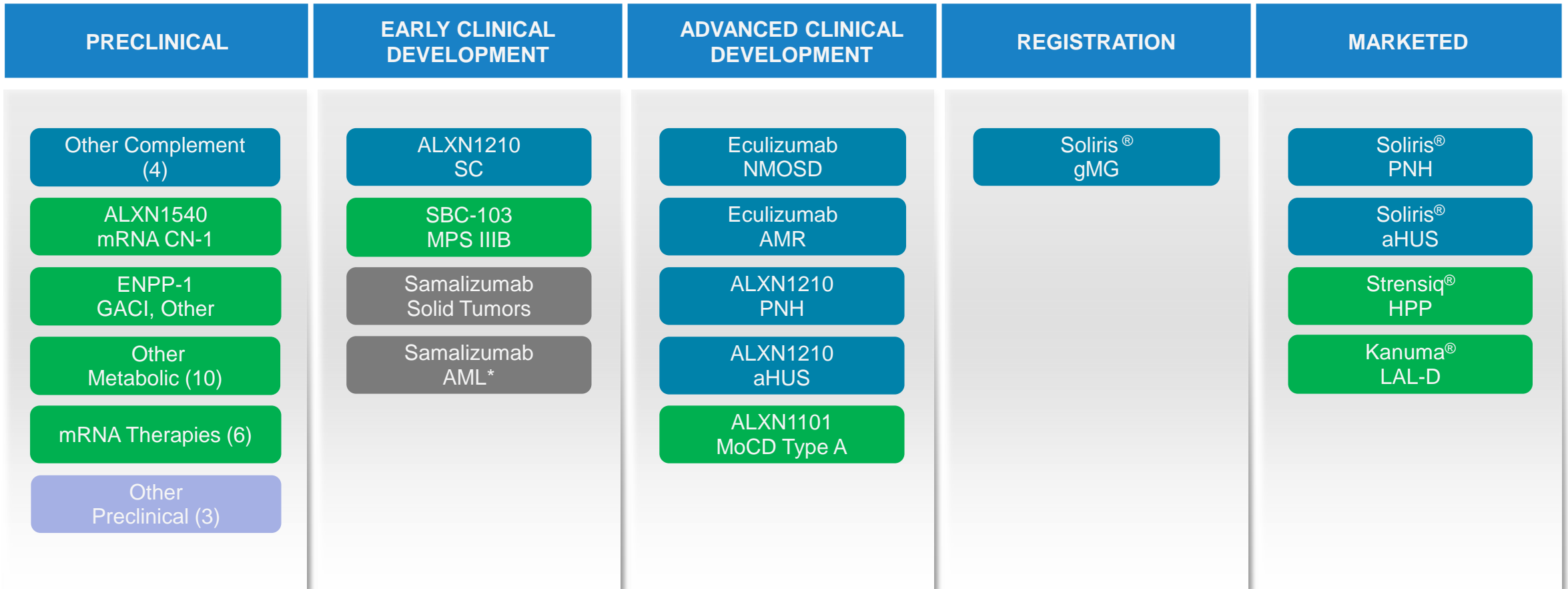


**>15**

**PRECLINICAL  
PROGRAMS**

In development

# Our Research Engine Driving Innovation



■ Complement   
 ■ Metabolic   
 ■ Immuno-Oncology   
 ■ Other

\*Part of the Leukemia and Lymphoma Society BEAT AML Master study;

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# 2017 Milestones

PROGRAM	INDICATION	STATUS	
Soliris	Refractory gMG	US, EU, and Japan Regulatory Submissions	Complement Portfolio
Soliris	Refractory gMG	US, EU, and Japan Regulatory Decisions	
Eculizumab	Relapsing NMOSD	Registration Trial – Complete Enrollment	
ALXN1210	PNH	Registration Trial – Complete Enrollment	
ALXN1210	aHUS	Registration Trial – Complete Enrollment Initiate Enrollment in Pediatric Study	
ALXN1210 SC	Multiple	Phase 1 – Progress Development	
Novel Complement Inhibitor	Multiple	Initiate Phase 1 Study	
SBC-103	MPS IIIB	Phase 1/2 – 6 and 12 Month Neurocognitive Data	Metabolic Portfolio
ALXN1101 cPMP	MoCD Type A	Pivotal Study – Progress Enrollment	
Samalizumab	Solid Tumors	Phase 1 – Progress Study	Immuno-Oncology
Samalizumab	AML*	Phase 1 – Progress Study	

\*Part of the Leukemia and Lymphoma Society BEAT AML Master Study

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# Growing Our Leadership in Rare Disease



## Grow

Advancing leadership in complement biology with IP, new indications & multiple complement inhibitors



## Build

Leveraging proven commercial strategy to establish metabolic franchise and drive diversification



## Expand

Investing in robust pipeline to deliver breakthrough innovation in multiple therapeutic areas



**DAVID BRENNAN**  
**INTERIM CEO**

**DAVE ANDERSON**  
**CFO**

Fireside Chat  
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