Alexion to Acquire Wilson Therapeutics

Conference Call

April 11, 2018

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Available for Q&A	Brian Goff, Chief Commercial Officer

Forward-Looking Statements

This presentation contains forward-looking statements, including statements related to guidance regarding anticipated financial results for 2018, the status of and plans for clinical trials for WTX101, the potential benefits of WTX101 for the treatment of Wilson disease, and the potential of the transaction to create shareholder value. 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 the adequacy of our research, 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, 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 results of our products in broader patient populations, the possibility that clinical trials of our product candidates could be delayed, the adequacy of our pharmacovigilance and drug safety reporting processes, 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 anticipated regulatory filings are delayed, the risk that estimates regarding the number of patients with Wilson disease are inaccurate, the risks of changing foreign exchange rates, risks relating to the potential effects of the Company's restructuring and relocation of its corporate headquarters, 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 Annual Report on Form 10-K for the period ended December 31, 2017 and in our other filings with the SEC. 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.

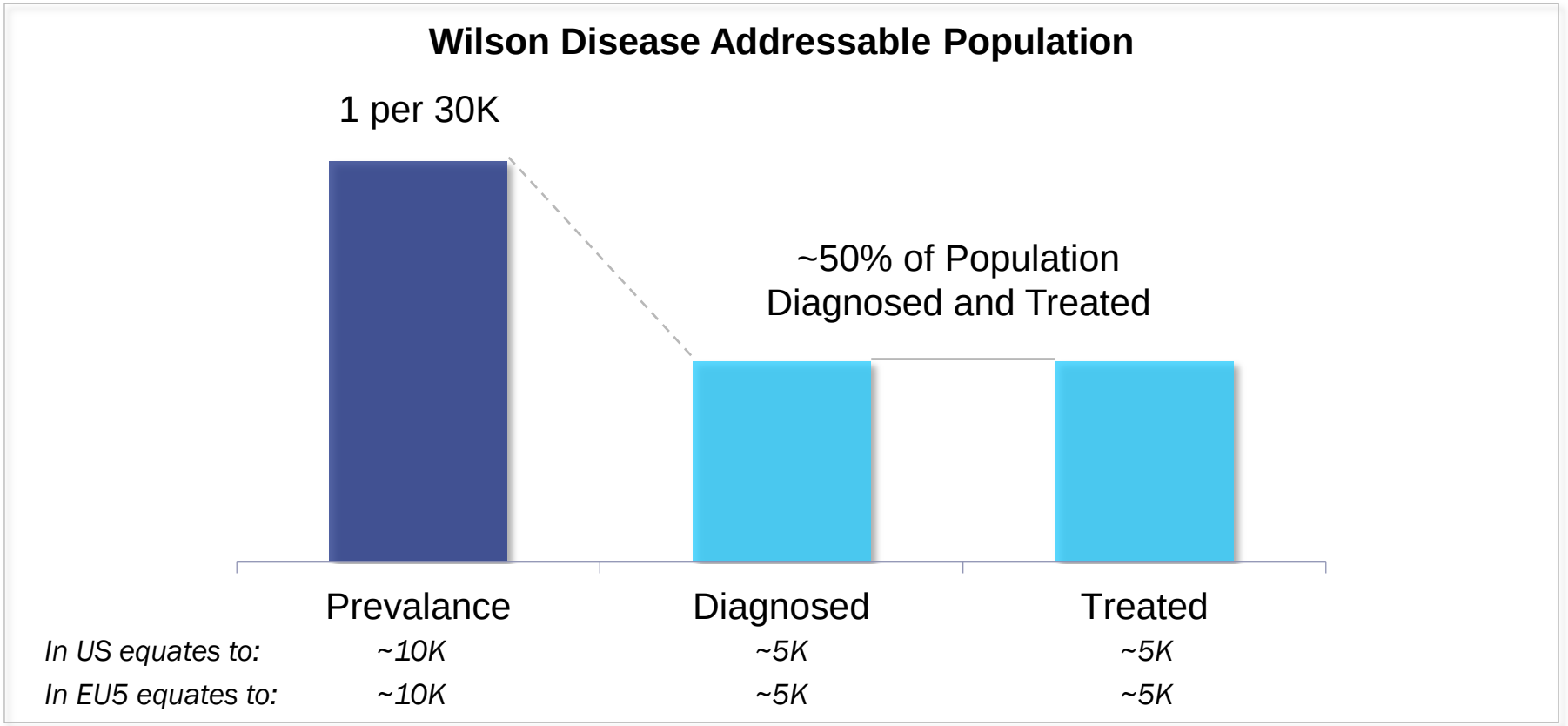
Summary & Strategic Rationale

Ludwig Hantson, Ph.D.
Chief Executive Officer

Wilson Therapeutics: Strong Strategic Fit, Builds Clinical Stage Pipeline in Rare Metabolic Disease

- Wilson disease is a rare, chronic, debilitating genetic disorder in which copper accumulates in vital organs, especially the liver and brain
- Existing treatments have significant efficacy, safety, and dosing limitations and have not been shown to address the underlying cause of disease
- WTX101, a first-in-class high affinity copper-protein-binding agent, is the first innovation in decades and has the potential to define a new standard of care for Wilson disease
  - Pre-clinical data have shown WTX101 has potential to de-copper the liver
  - Positive POC Phase 2 data showed WTX101 can reduce and control free copper levels and improve symptoms and associated disabilities
  - Pivotal Phase 3 trial enrollment began February 2018
- Builds the clinical stage pipeline
- Leverages expertise and commercial capabilities in metabolic and neurologic diseases
- Consistent with our disciplined capital allocation approach and business development goals for 2018

Roughly 10,000 Treated Patients in the US and EU5; Differentiated Profile of WTX101 Can Serve Significant Unmet Needs



WTX101 has potential to better serve patients by addressing significant unmet needs over current standard of care including removing copper from the liver and reducing the risk of neurological deficits

Sources: Gomes, 2016; Poujois, 2017; Merle, 2007, Beinhardt, 2014

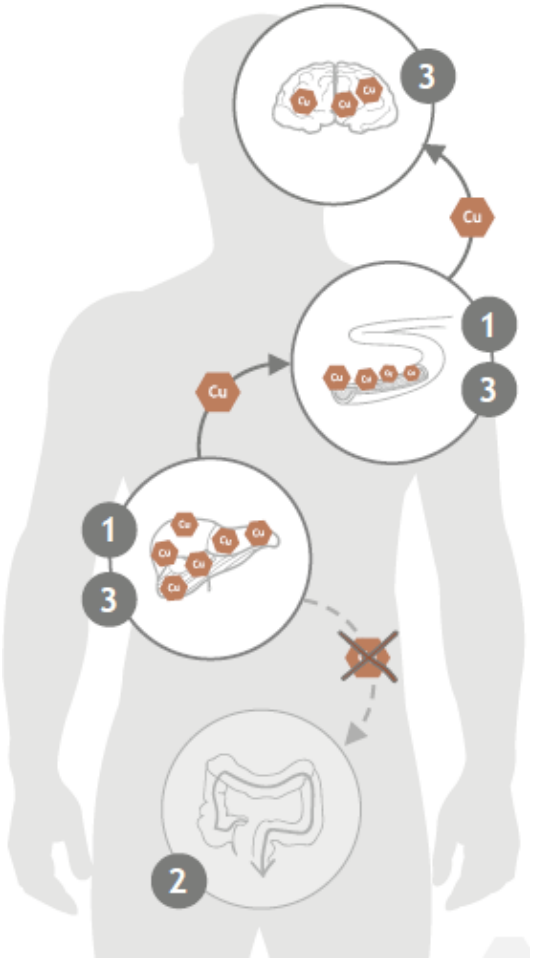
Overview of Wilson Disease and WTX101

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

Wilson Disease is a Rare, Severe Metabolic Disorder

- Rare genetic disorder of impaired copper (Cu) transport with devastating hepatic and neurological consequences that requires life-long therapy
- Caused by mutations in the ATP7B gene that leads to excess copper accumulating in liver cells and increased levels of toxic free copper
- Failure to clear hepatic copper can have detrimental effects:
  - Jaundice, fluid retention, confusion and synthetic liver dysfunction increased risk of cirrhosis, liver failure, and liver cancer
  - Fatigue, pain, swelling, vomiting and upper gastrointestinal bleeding
- Neurologic worsening can be severe:
  - Significant neurological morbidity including problems with movement, gait, speech, and swallowing
  - Psychiatric disorders including depression, mania, irritability, psychosis and personality changes

Copper (Cu) balance is normally maintained in the body by hepatic excretion of excessive copper in bile. In Wilson disease there is:



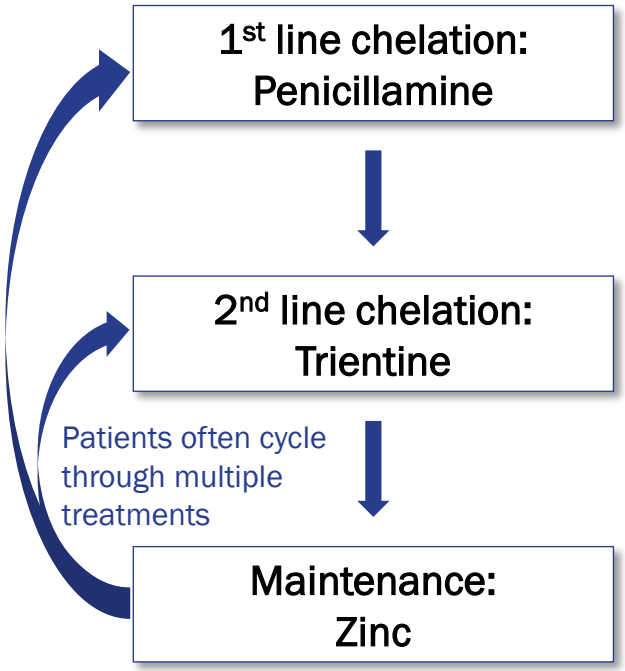
1. Diminished loading of Cu onto the ceruloplasmin (Cp) protein in liver leads to suboptimal transport of Cp-bound Cu into blood
2. Diminished excretion of excess Cu through the bile into feces
3. *Increased levels of free Cu (toxic) in blood, liver, brain and other organs*

Alexion's Expertise in Rare Disease To Improve the Treatment Paradigm

	Treatment Paradigm
Presentation	■ Patients generally present to the PCP and are referred to the hepatologist or neurologist ■ Hepatologist: Elevated liver enzymes/symptoms; may go to community hep before specialized hep ■ Neurologist: Neurologic symptoms; may go to psychiatrist before neurologist
Diagnosis	■ A combination of tests are used to confirm a diagnosis of Wilson disease, the most common being liver enzymes and serum or urine copper ■ As disease manifestations are not exclusive to Wilson disease and disease awareness is low, patients can experience multi-year delays before getting an accurate diagnosis
Ongoing Care	■ Patients are most commonly managed by hepatologists and neurologists at Wilson disease Centers of Excellence

Limitations of Current Treatments

Treatment Paradigm



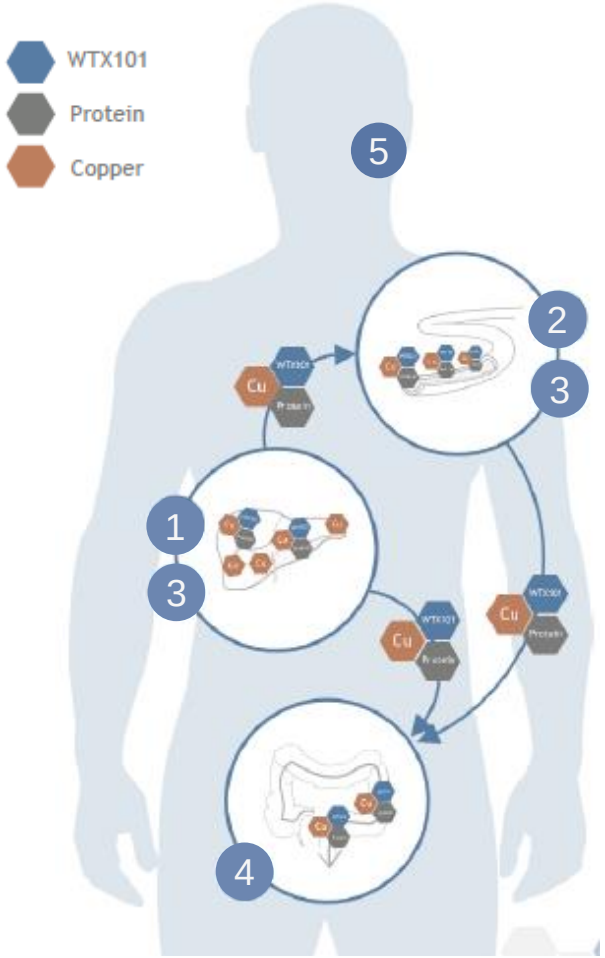
Limitations of Chelators / Zinc

1. Low affinity to Cu
2. Not specific to Cu
3. Does not form stable tripartite complexes with proteins
4. Cu not excreted through bile into feces
5. Complex dosing regimen

Limitations Lead to Significant Unmet Needs :

Effective copper control, in particular removal from the liver | Reduced risk of drug-induced neurological worsening
Improved safety and tolerability | Simplified dosing regimen for improved compliance

WTX101: Novel First-in-Class Small Molecule with Unique MOA



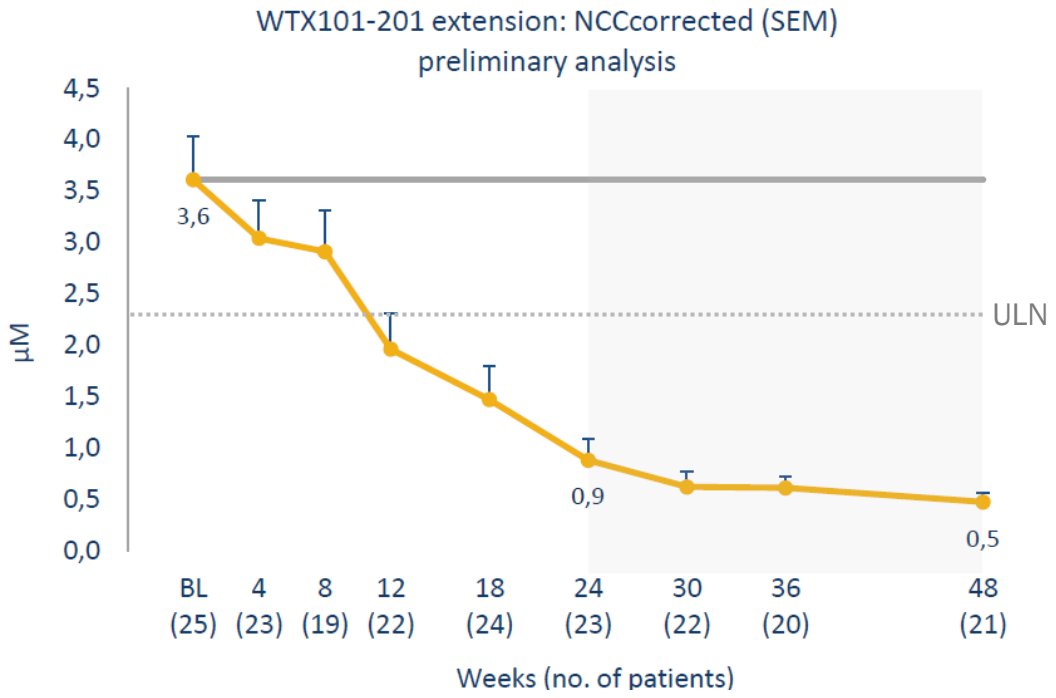
WTX101:
(bis-choline tetrathiomolybdate)

1	High affinity to Cu	10,000-fold higher affinity for Cu than chelators, allowing for ***removal from intracellular stores in the liver***
2	Specific to Cu	Specifically binds Cu, not other metals (Zn, Fe, Ca, Mn, Mg) typically associated with treatment side effects
3	Forms stable tripartite complexes with proteins	Safe Cu transport in the blood, ***reducing the risk of drug induced neurological deficits***
4	Excretion of Cu through bile into feces	Excretes excess Cu via natural route ***limiting potential nephrotoxicity***
5	Simplified dosing regimen	***Oral, once daily dosing*** and rapid onset of action

Phase 2 Data Demonstrated Strong Proof-of-Concept

Significant mean reduction of serum free copper

- 72% reduction from baseline to week 24 in free serum copper ($p < 0.0001$)

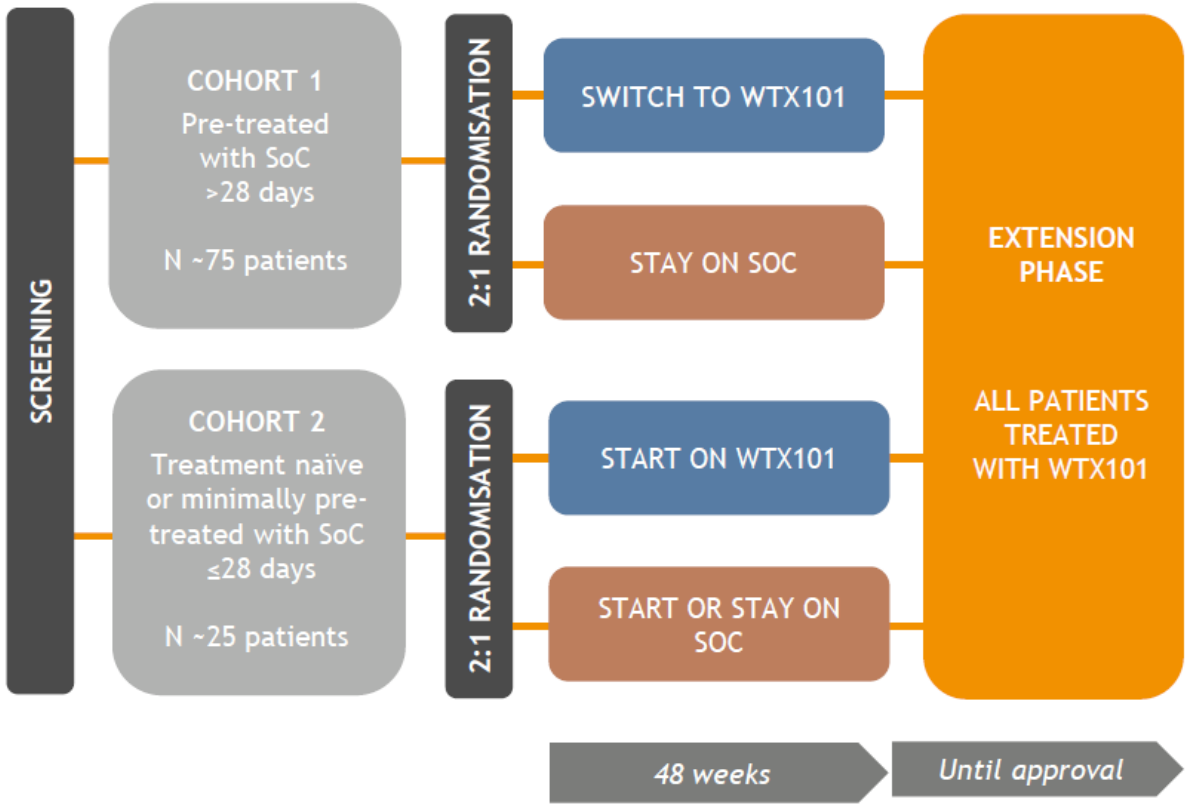


- Mean free copper levels were reduced to level below upper limit of normal (2.3 µM) after 12 weeks
- Effect sustained at 48 weeks in the extension study
- Liver status stabilized or improved in majority of patients
- WTX101 also demonstrated improvement in patient reported disability and neurological status
- WTX101 was generally well tolerated
- Most reported AEs were mild or moderate in intensity

Source: Wilson Therapeutics; Phase 2 study results published in Lancet Gastroenterol Hepatol 2017

Phase 3 Trial Began Enrollment in Q1

- ~100 patients with neurologic and hepatic symptoms, randomized 2:1 (WTX101:SoC)
- 2 cohorts
  - Pre-treated with SoC >28 days
  - Treatment naïve or minimally pre-treated with SoC ≤28 days
- Non-inferiority vs. SoC
  - 15% NI-margin
  - Superiority will be assessed if NI met
- Primary endpoint
  - Cu control ($NCC_{corrected}$, as in Phase 2)
- Additional endpoints include
  - Neurological and hepatic function
  - Safety/tolerability
  - Quality of life



WTX101 has orphan drug designation in the US and EU and FDA Fast Track status

Intend to enhance the clinical development plan to further differentiate WTX101

Financial Overview

Paul Clancy
Chief Financial Officer

Acquisition Terms & Financial Impact

Acquisition Terms

- Acquisition through a public tender offer under Swedish Takeover Act and Nasdaq Stockholm Takeover Rules
- Offer price of SEK 232 in cash for each share in Wilson Therapeutics, totaling approximately $855M USD
- Subject to regulatory approval

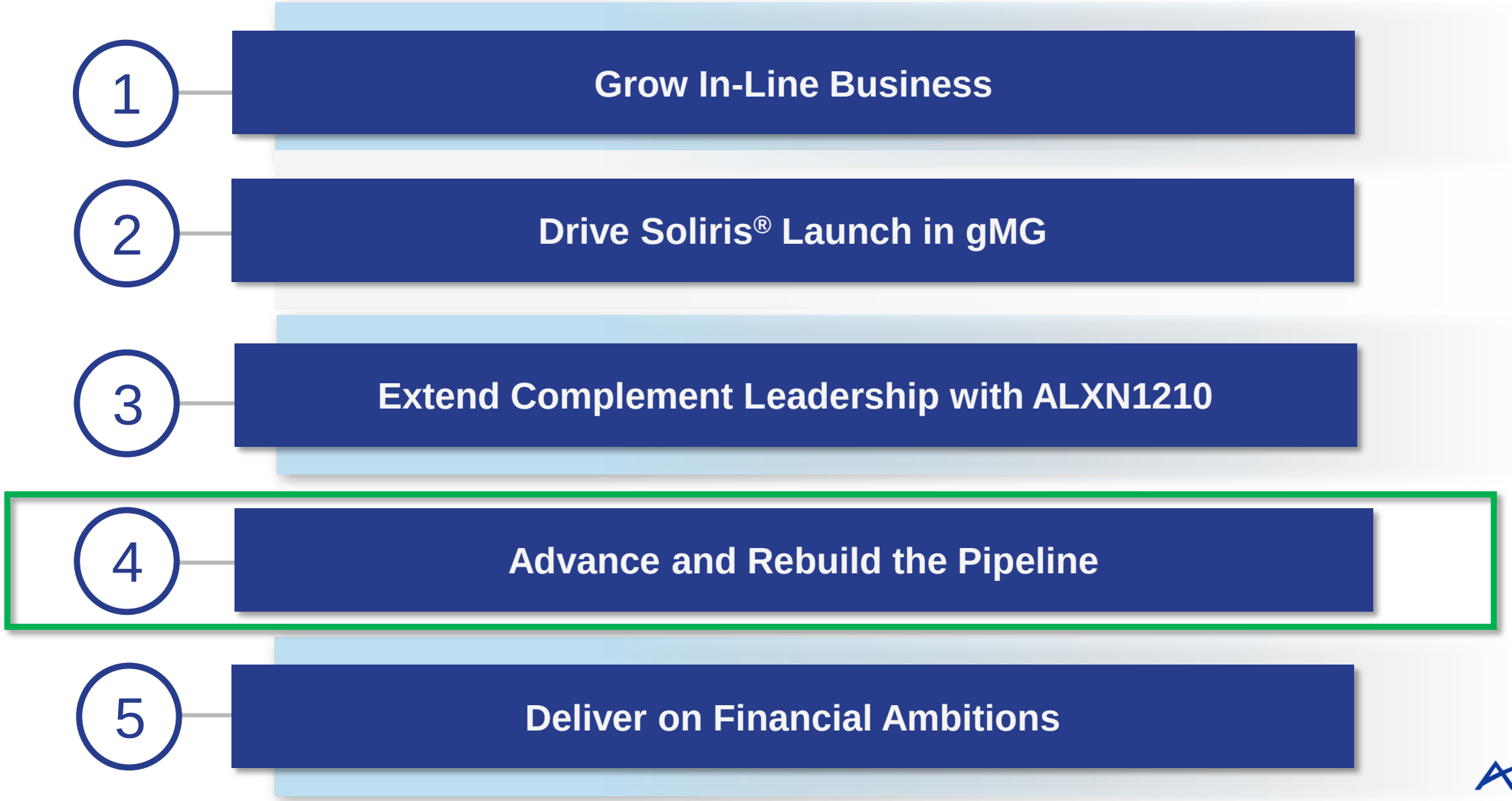
Financial Impact

- Financed using cash
- Closing expected Q2 2018
- Following the closing, acquisition cost will be expensed immediately to R&D on GAAP P&L
- Associated expenses for 2018 and 2019 covered by BD R&D expense included in guidance provided in February
- Retain capacity to continue to build the pipeline

Closing Remarks

Ludwig Hantson, Ph.D.
Chief Executive Officer

2018 Key Initiatives to Drive Sustainable Long Term Growth



Provided April 11, 2018, as part of an oral presentation and is qualified by such, contains forward-looking statements, actual results may vary materially; Alexion disclaims any duty to update.