

# Rare Disease

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# Forward looking statements

In order, among other things, to utilise the 'safe harbour' provisions of the US Private Securities Litigation Reform Act of 1995, AstraZeneca (hereafter 'the Group') provides the following cautionary statement: This document contains certain forward-looking statements with respect to the operations, performance and financial condition of the Group, including, among other things, statements about expected or targeted revenues, margins, earnings per share or other financial or other measures (including the Financial Ambition Statements described in this presentation). Although the Group believes its expectations and targets are based on reasonable assumptions and has used customary forecasting methodologies used in the pharmaceutical industry and risk-adjusted projections for individual medicines (which take into account the probability of success of individual clinical trials, based on industry-wide data for relevant clinical trials at a similar stage of development), any forward-looking statements, by their very nature, involve risks and uncertainties and may be influenced by factors that could cause actual outcomes and results to be materially different from those predicted. The forward-looking statements reflect knowledge and information available at the date of preparation of this document and the Group undertakes no obligation to update these forward-looking statements. The Group identifies the forward-looking statements by using the words 'anticipates', 'believes', 'expects', 'intends' and similar expressions in such statements. Important factors that could cause actual results to differ materially from those contained in forward-looking statements, certain of which are beyond the Group's control, include, among other things: the risk of failure or delay in delivery of pipeline or launch of new medicines; the risk of failure to meet regulatory or ethical requirements for medicine development or approval; the risk of failures or delays in the quality or execution of the Group's commercial strategies; the risk of pricing, affordability, access and competitive pressures; the risk of failure to maintain supply of compliant, quality medicines; the risk of illegal trade in the Group's medicines; the impact of reliance on third-party goods and services; the risk of failure in information technology or cybersecurity; the risk of failure of critical processes; the risk of failure to collect and manage data in line with legal and regulatory requirements and strategic objectives; the risk of failure to attract, develop, engage and retain a diverse, talented and capable workforce; the risk of failure to meet regulatory or ethical expectations on environmental impact, including climate change; the risk of the safety and efficacy of marketed medicines being questioned; the risk of adverse outcome of litigation and/or governmental investigations; intellectual property-related risks to the Group's products; the risk of failure to achieve strategic plans or meet targets or expectations; the risk of failure in financial control or the occurrence of fraud; the risk of unexpected deterioration in the Group's financial position; the impact that global and/or geopolitical events may have, or continue to have, on these risks, on the Group's ability to continue to mitigate these risks, and on the Group's operations, financial results or financial condition. There can be no guarantees that the conditions to the closing of the proposed transaction with Fusion will be satisfied on the expected timetable, or at all, or that "FPI-2265" (Ac225-PSMA I&T) or any combination product will receive the necessary regulatory approvals or prove to be commercially successful if approved. There can be no guarantees that the conditions to the closing of the proposed transaction with Amolyt Pharma will be satisfied on the expected timetable, or at all, or that eneboparatide ('AZP-3601') will receive the necessary regulatory approvals or prove to be commercially successful if approved.

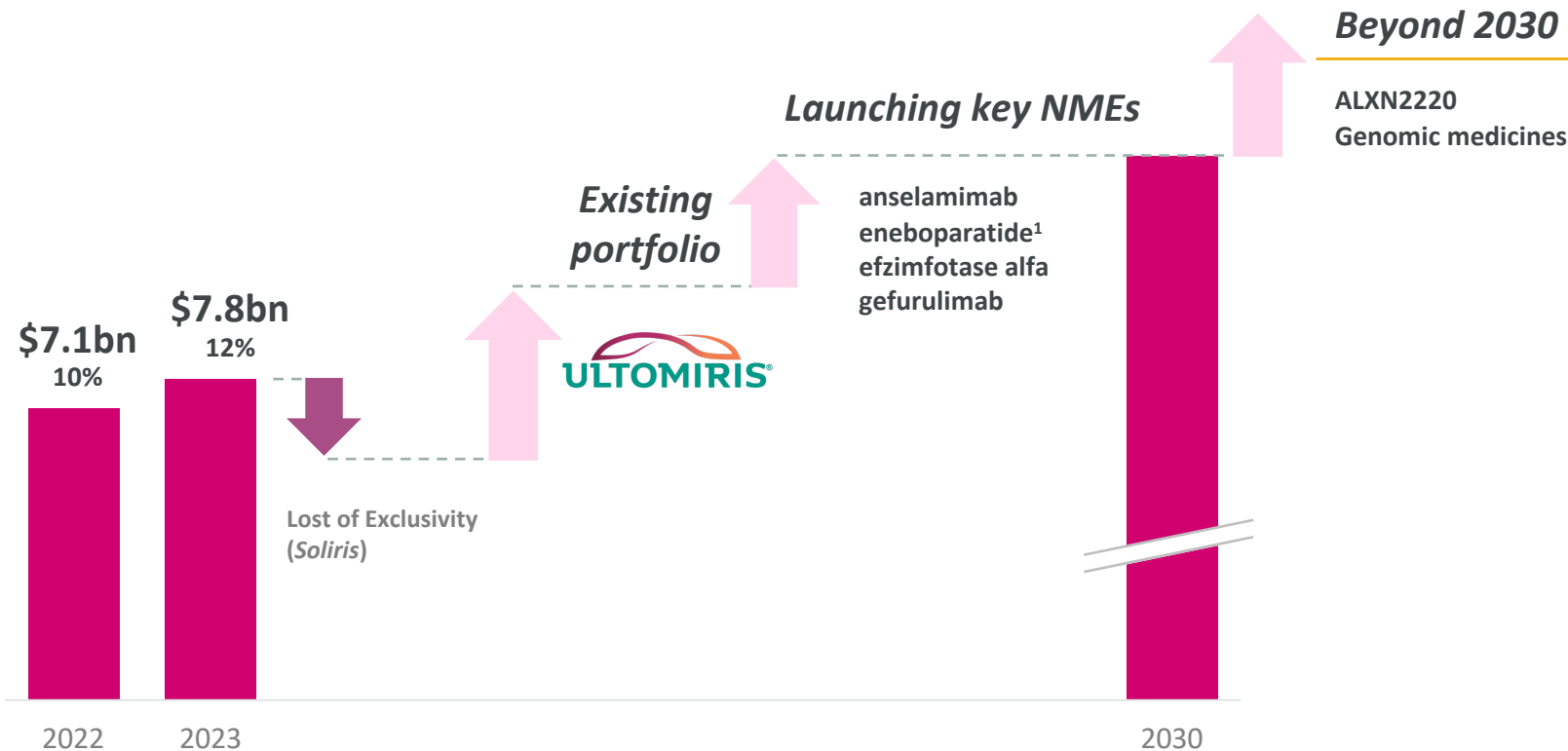
This presentation includes references to new molecular entities and life-cycle management programmes that are being investigated in current or future clinical trials, and as such have not been approved by any regulatory agency. For a list of new molecular entities and indications in development, see pages 7-11 of the Clinical Trials Appendix that accompanied AstraZeneca's Q1 2024 results.

## Basis of AstraZeneca ambitions, forecasts and targets

AstraZeneca ambitions, forecasts and targets in this presentation (the "Financial Ambition Statements") are derived from AstraZeneca's most recent risk-adjusted mid- and long-term plans, adjusted for developments in the business since those plans were finalised. Financial Ambition Statements presented are based on management's risk-adjusted projections for individual medicines and individual clinical trials. Estimates for these probabilities are based on industry-wide data for relevant clinical trials in the pharmaceutical industry at a similar stage of development adjusted for management's view on the risk profile of the specific asset. The peak year revenue (PYR) potential for individual medicines referred to in this presentation are the maximum estimated Total Revenue to be recognised by AstraZeneca in a single calendar year, during the lifecycle of the medicine, and are based on management's latest non-risk adjusted forecast estimates. Estimates are based on customary forecasting methodologies used in the pharmaceutical industry. Peak year revenue may occur in different years for each NME depending on trial outcomes, approval label, competition, launch dates and exclusivity periods, amongst other variables. The peak year revenue figures are derived from net sales at nominal values and are not risk-adjusted or time-value discounted. The development of pharmaceutical products has inherent risks given scientific experimentation and there are a range of possible outcomes in clinical results, safety, efficacy and product labelling. Clinical results may not achieve the desired product profile and competitive environment, pricing and reimbursement may have material impact on commercial revenue forecasts. By their nature, forecasts are based on a multiplicity of assumptions and actual performance in future years may vary, significantly and materially, from these assumptions. The Financial Ambition Statements in this presentation are based on Q1 2024 exchange rates; AZ undertakes no obligation to update those statements based on future currency movements

# Rare Disease – next wave of growth to 2030 and beyond

Illustrative only, not to scale



**Complement**  
continued leadership

**Beyond Complement**  
focused on first- and/or  
best-in-class medicines

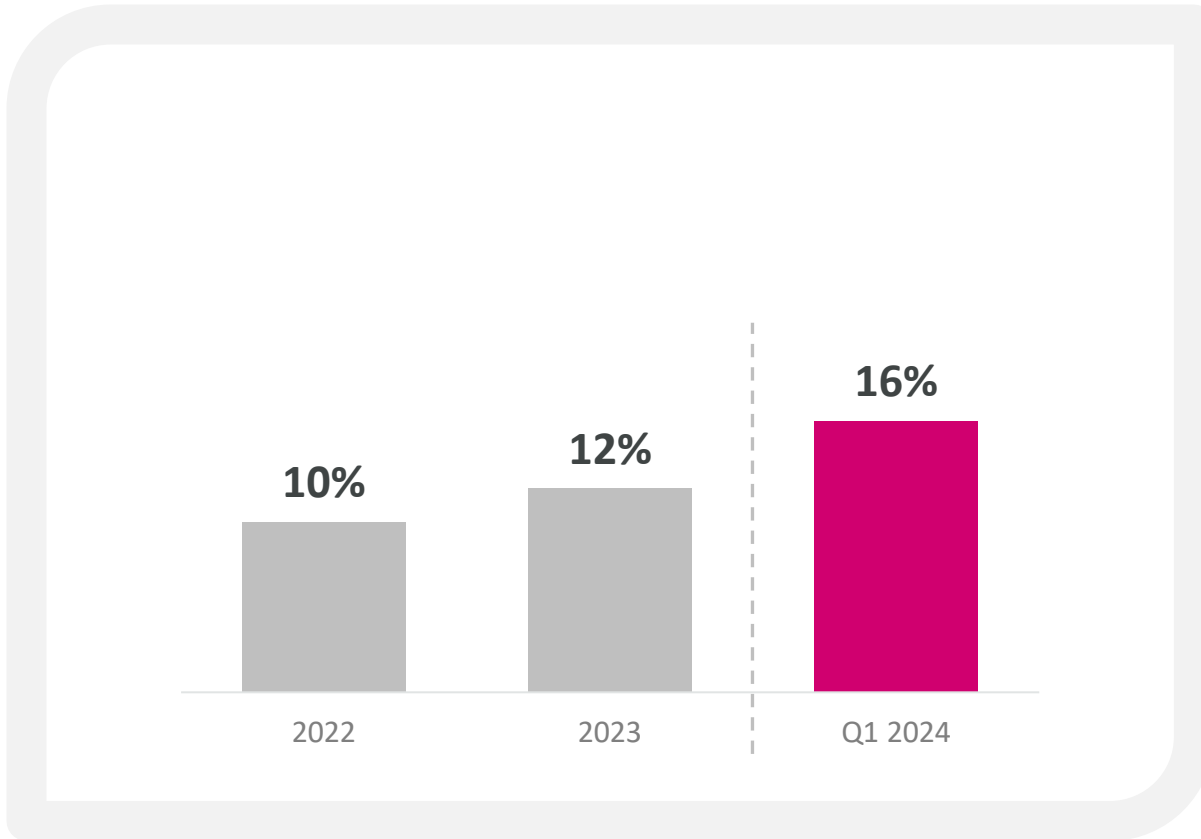
**Technologies**  
investing in new, potentially  
curative, modalities

FY 2022 growth rates on medicines acquired with Alexion have been calculated on a pro forma basis comparing to the corresponding period in the prior year. In FY 2022 Total Revenue from *Koselugo* is included in Rare Disease (FY 2021: Oncology) and Total Revenue from *Andexxa* is included in BioPharmaceuticals: CVRM (FY 2021: Rare Disease). The growth rate shown has been calculated as though these changes had been implemented in FY 2021.

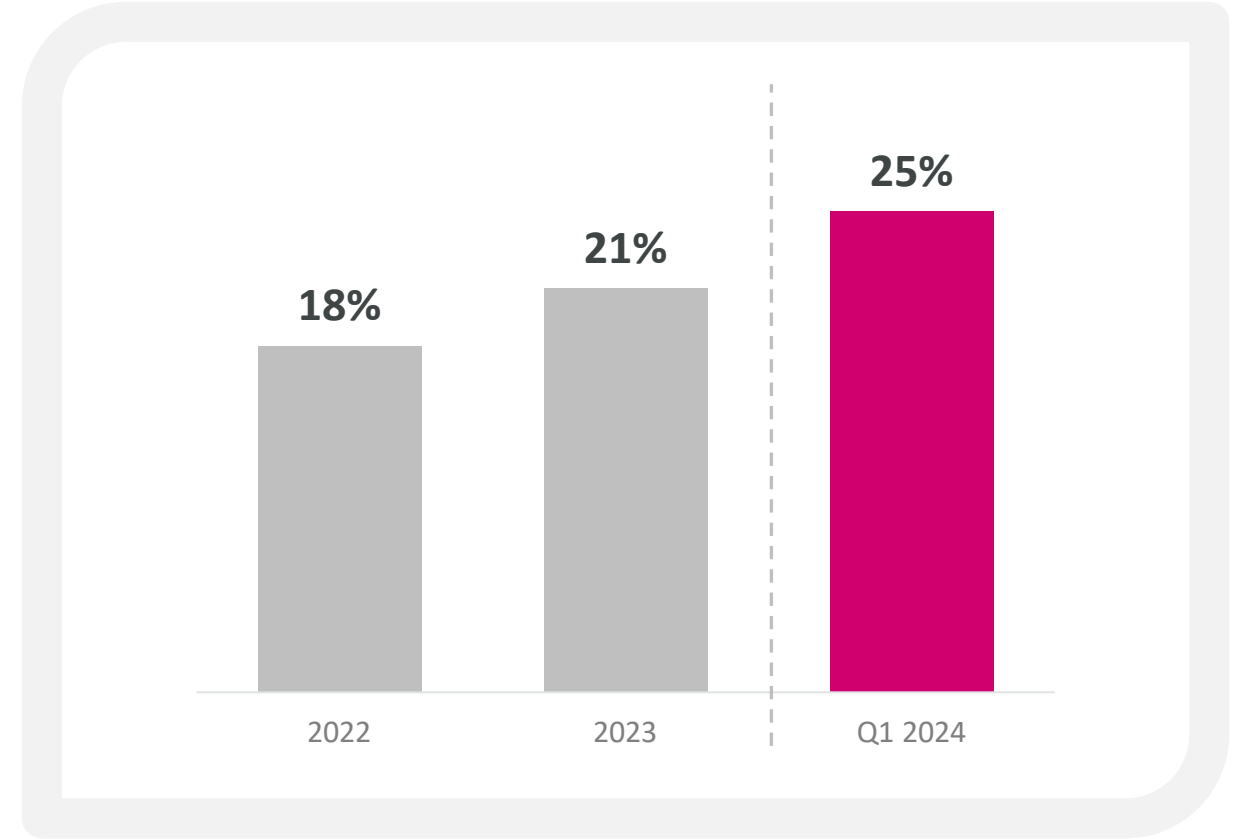
1. Amolyt acquisition remains subject to customary external clearances; all clinical development plans mentioned herein subject to deal closure. All growth rates at CER. Acronym definitions can be found in Glossary.

# Alexion has delivered sustainable and robust growth since acquisition

## Rare Disease Total Revenue growth (%)



## Rare Disease patient growth (%)

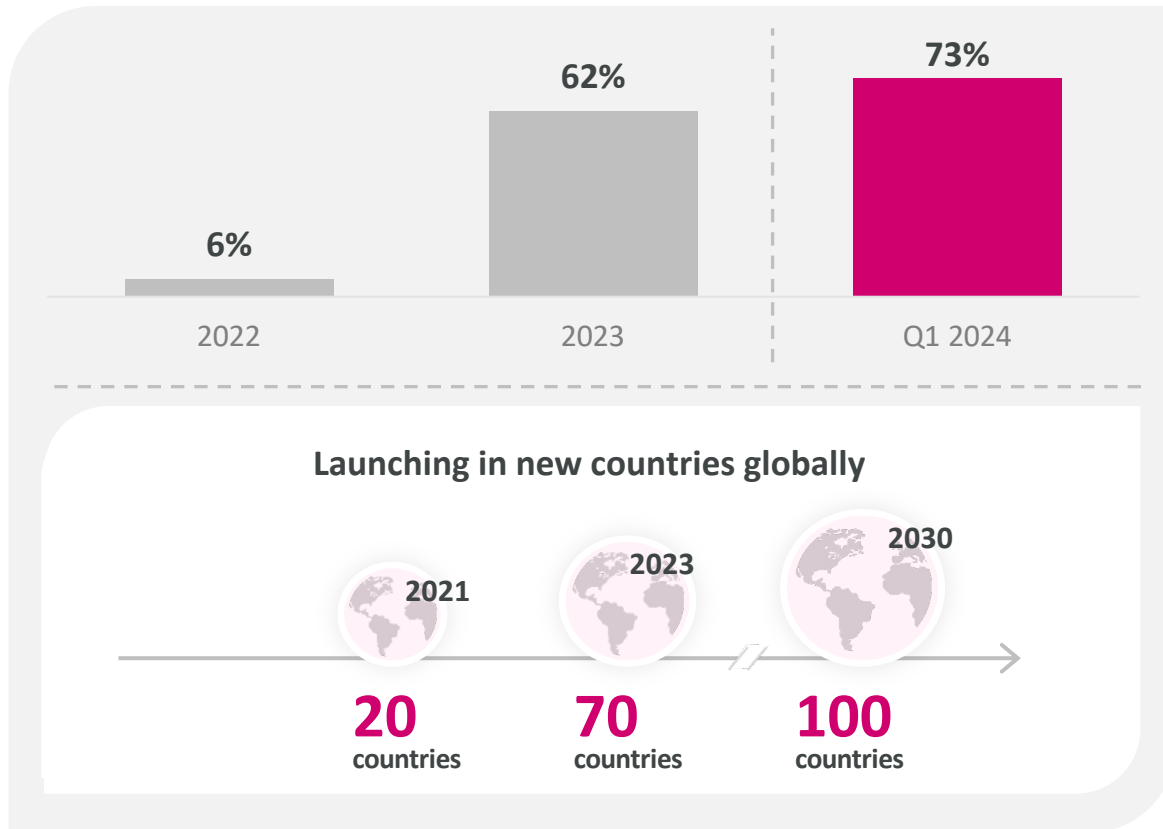


FY 2022 growth rates on medicines acquired with Alexion have been calculated on a pro forma basis comparing to the corresponding period in the prior year. In FY 2022 Total Revenue from *Koselugo* is included in Rare Disease (FY 2021: Oncology) and Total Revenue from *Andexxa* is included in BioPharmaceuticals: CVRM (FY 2021: Rare Disease). The growth rate shown has been calculated as though these changes had been implemented in FY 2021. All growth rates at CER.

4 Acronym definitions can be found in Glossary.

# Emerging Markets growth leveraging AstraZeneca global footprint

## Emerging Markets Total Revenue growth (%)



## Strategic opportunity in China

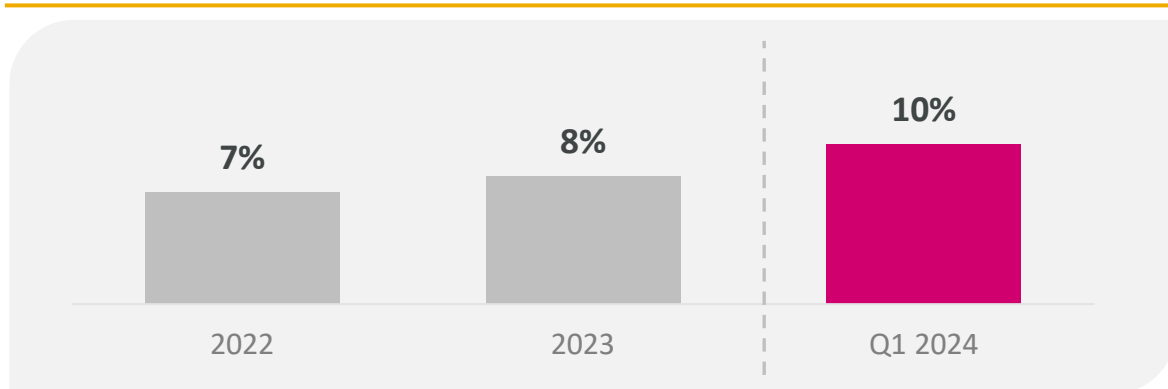


5 FY 2022 growth rates on medicines acquired with Alexion have been calculated on a pro forma basis comparing to the corresponding period in the prior year. In FY 2022 Total Revenue from *Koselugo* is included in Rare Disease (FY 2021: Oncology) and Total Revenue from *Andexxa* is included in BioPharmaceuticals: CVRM (FY 2021: Rare Disease). The growth rate shown has been calculated as though these changes had been implemented in FY 2021. All growth rates at CER. Acronym definitions can be found in Glossary.

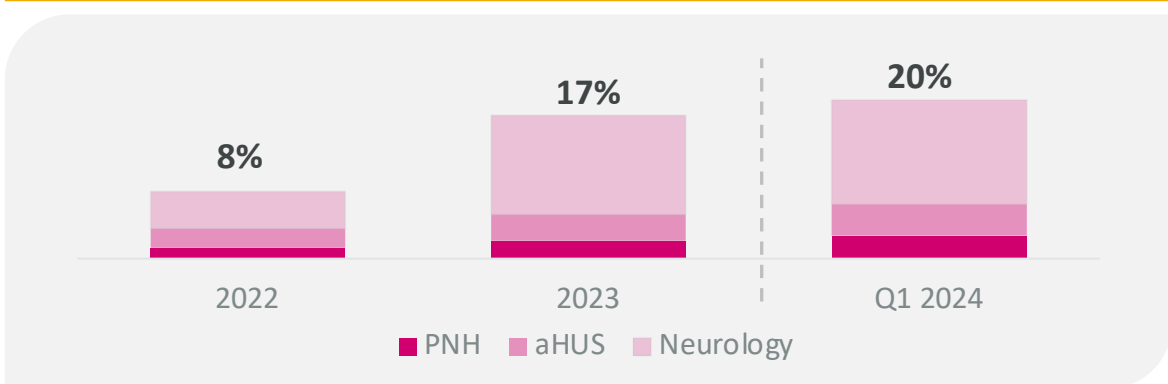
# Complement

# Sustainable growth of C5 Franchise

## C5 Total Revenue growth (%)



## C5 patient growth by indication (%)



**Rapid conversion** from *Soliris* to *Ultomiris*



**Superior efficacy** with rapid, sustained complement inhibition and steroid sparing data



**Geographic reach** leveraging well-established AstraZeneca network

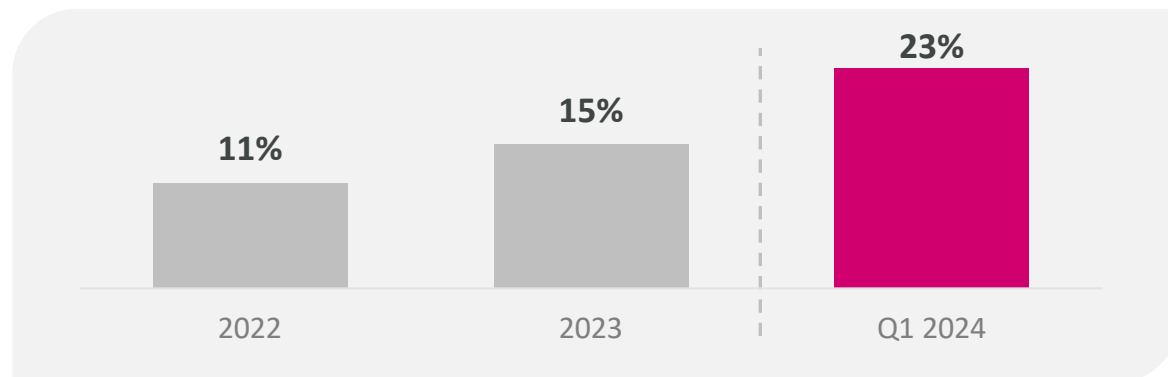


**Indication expansion** to maximise complement-mediated and adjacent diseases

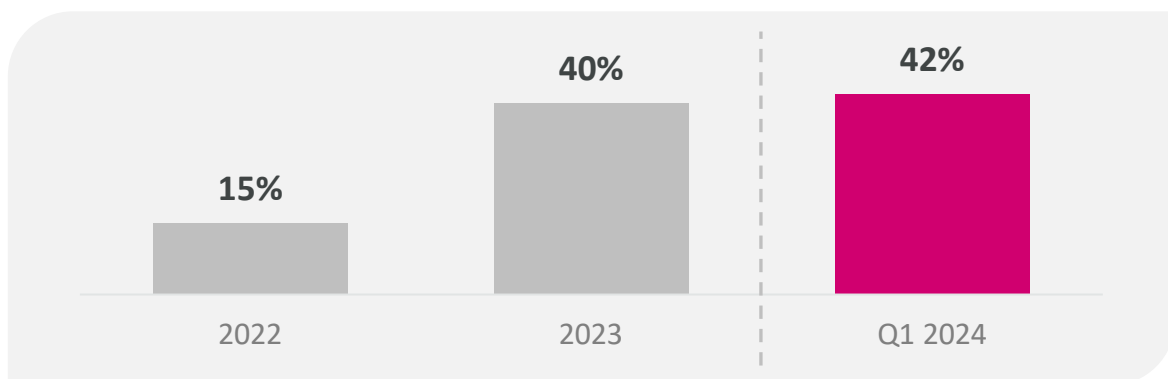
\*Peak Year Revenues, non-risk adjusted. FY 2022 growth rates on medicines acquired with Alexion have been calculated on a pro forma basis comparing to the corresponding period in the prior year. Neurology includes gMG and NMOSD. In FY 2022 Total Revenue from *Koselugo* is included in Rare Disease (FY 2021: Oncology) and Total Revenue from *Andexxa* is included in BioPharmaceuticals: CVRM (FY 2021: Rare Disease). The growth rate shown has been calculated as though these changes had been implemented in FY 2021. All growth rates at CER. Acronym definitions can be found in Glossary.

# Neurology driving C5 growth in the short- to mid-term

## Neurology Total Revenue growth (%)<sup>1</sup>



## Neurology patient growth (%)



### Sustained long-term improvements

88% of *Ultomiris* patients saw clinically meaningful improvements in activities of daily living to Week 164<sup>2</sup>

**Steroid sparing data** >75% of *Soliris* patients were on low dose steroids after 2 years<sup>3</sup>

**Vs competition (FcRN)** switch dynamic has consistently favoured *Ultomiris* since Q3 2023<sup>4</sup>

**Long-term evidence of superior efficacy in gMG**

1. Internal estimated growth rates. 2. Vu et al. AAN 13-18 April 2024 Long term efficacy and safety of ravulizumab, a long-acting terminal complement inhibitor, in adults with AChR Ab+ generalized myasthenia gravis: Final results from the Phase 3 CHAMPION MG open-label extension. 3. Pulley M. et al., AANEM 2023 Annual Meeting, November 1-4, 2023, Phoenix, AZ, USA. 4. Based on raw claims data from Komodo Prism 17 April 2024. Neurology includes gMG and NMOSD.



# gMG – expanding reach with next-generation gefurulimab

Opportunity to treat earlier and broader patient population<sup>1</sup>



Q2W

**Soliris**

SoC for more severe, refractory patients



Q8W

**Ultomiris**

biologic of choice for broader population



QW

**gefurulimab**

(ALXN1720)

potential best-in-class self-administrative s.c. option

US diagnosed patients: **72,000** AChR+ across gMG MGFA Classes II to IV

66,000

36,000

9,000

corticosteroids

1<sup>st</sup> immuno-suppressant

2<sup>nd</sup> immuno-suppressant

3<sup>rd</sup> immuno-suppressant  
or IVig/PP/PLEX

or  
**Soliris**

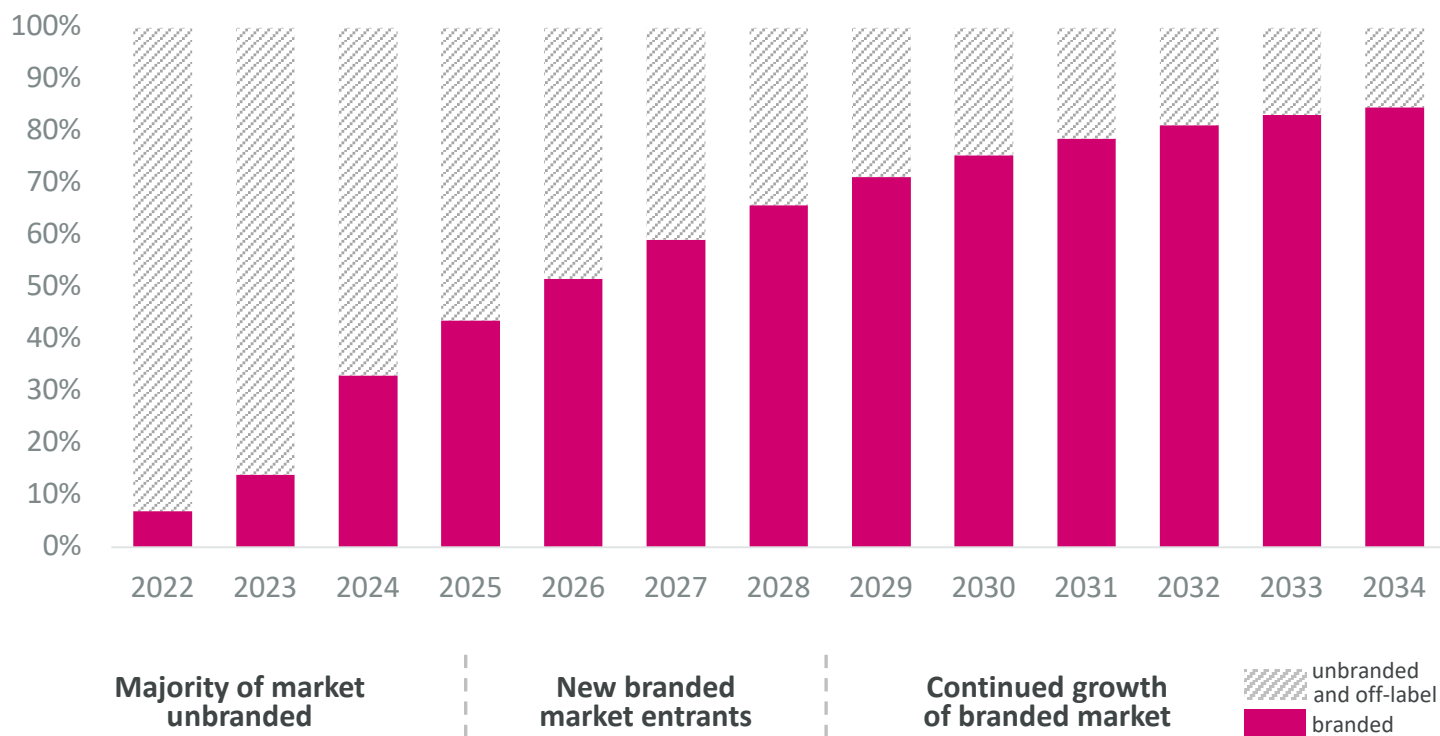
**Soliris**

**Ultomiris**

**gefurulimab (ALXN1720)**

# gMG – forecasted market evolution across Top 7 countries

gMG market evolution across Top 7 countries<sup>1</sup>



**80% of patients** to move to branded medicines by 2034<sup>2</sup>

gMG launched in 31 countries with **additional 25 countries** by 2025

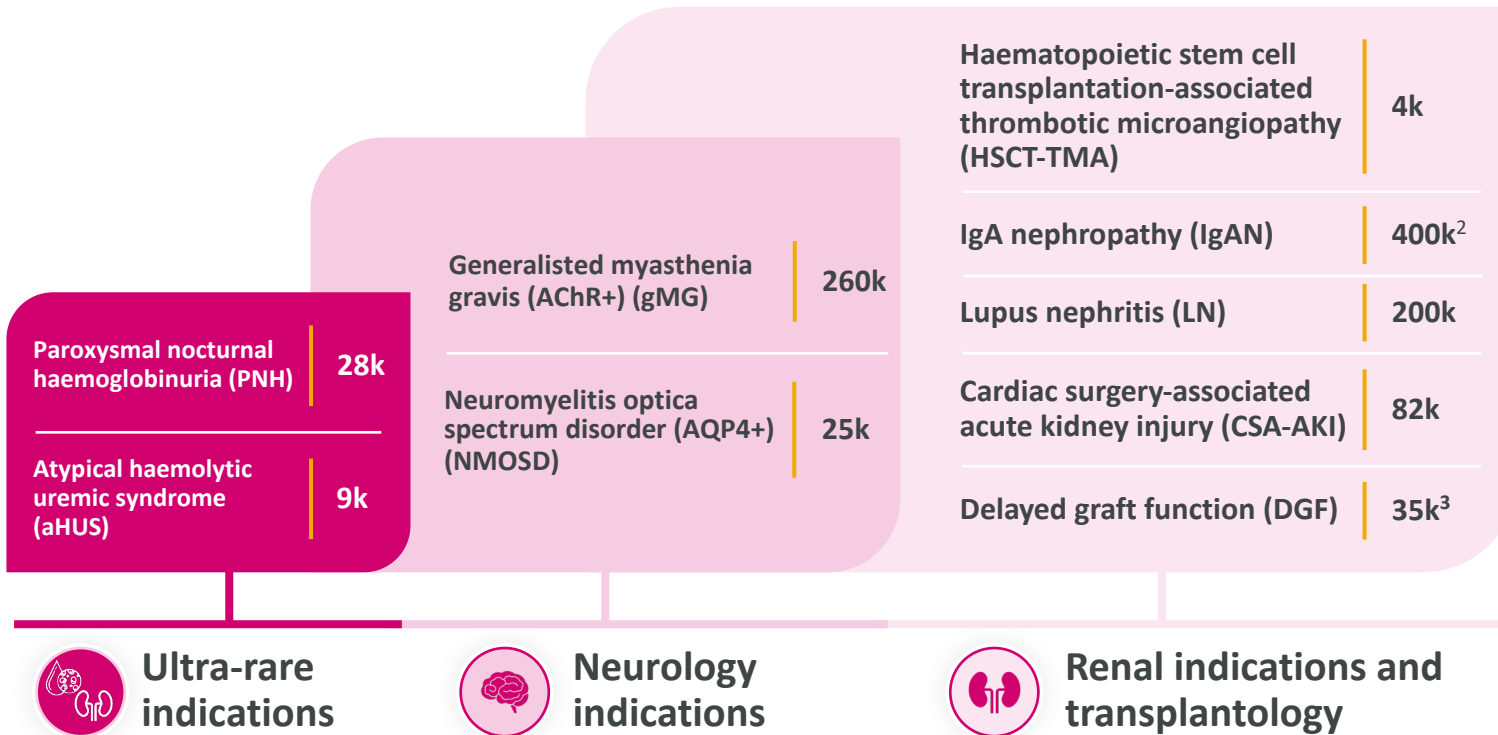
We anticipate patients treated with **Ultomiris, Soliris** and **gefurulimab** to grow significantly over the next 10 years

1. Top 7 = US, EU4 (Germany, France, Italy, Spain), Japan and Canada; based on market research and internal forecasts. 2. Branded products include non-steroid and non-immunosuppressants. 3. Excluding China, from 2024-2034. Acronym definitions can be found in Glossary.

# Ultomiris indication expansion, maximising complement-mediated and adjacent diseases

## Phase III near-term catalysts

Patients diagnosed<sup>1</sup>



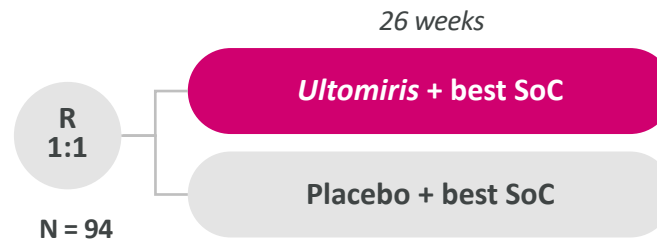
<b>TM-313/4</b> HSCT-TMA	<b>2025</b> data readout
<b>ARTEMIS</b> CSA-AKI	<b>&gt;2025</b> data readout
<b>ICAN</b> IgAN	<b>&gt;2025</b> data readout

\*Peak Year Revenue, non-risk adjusted. 1. Epidemiology reflect data for US, EU5 (Germany, France, UK, Italy, Spain), Japan and China. 2. Diagnosed IgAN patients excluding China. 3. Reflects deceased donor transplant patients.

# HSCT-TMA – potential for *Ultomiris* in rare, life-threatening complication of bone marrow transplant

Phase III trials ongoing

**Adult**  
TM-313



**Paediatric**  
TM-314



**Primary endpoints:**  
complete TMA response

**Key secondary endpoints:**  
haematologic response,  
overall survival, eGFR

Data anticipated  
**in 2025**

Factors associated with HSCT induce **dysregulation of the complement system**

**Adult and paediatric trials** advanced recruitment

Approximately **50k HSCT procedures** per year<sup>1</sup>

# IgAN – potential to transform course of disease with complement inhibition

## IgAN patient progression

**120k diagnosed**  
with IgAN in US

**Poor IgAN outcomes,**  
few patients avoid  
kidney failure<sup>1</sup>

**60-80% of high-risk  
patients** experienced  
kidney failure within  
10 years<sup>1</sup>

**First-line intervention**  
reduces renal functional decline

ACE inhibitor  
or ARB

+



ET<sub>AS</sub>

SoC

Post-SoC

Mild

**Complement inhibition**  
has potential to modify disease

**vemircopan**  
factor D inhibitor



**Ultomiris**  
C5 inhibitor



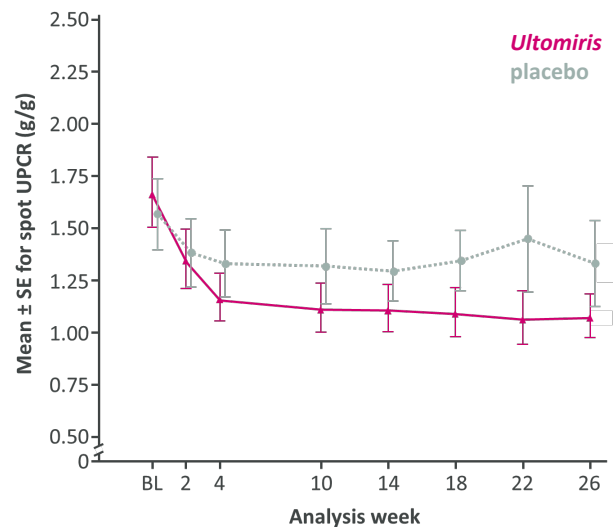
Residual high proteinuria:  
UPCR >0.75 g/g

Severe

# IgAN – rapid and sustained proteinuria reduction in Phase II supports Phase III trial

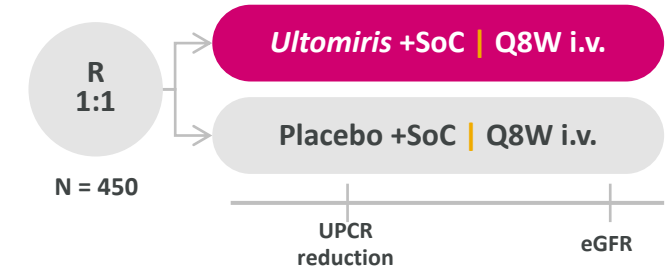
## Ultomiris Phase II SANCTUARY<sup>1</sup>

Mean spot UPCR over 26 weeks



- ✓ >40% reduction in proteinuria
- ✓ Stable eGFR
- ✓ *Ultomiris* ICAN Phase III trial initiated

## Ultomiris Phase III ICAN



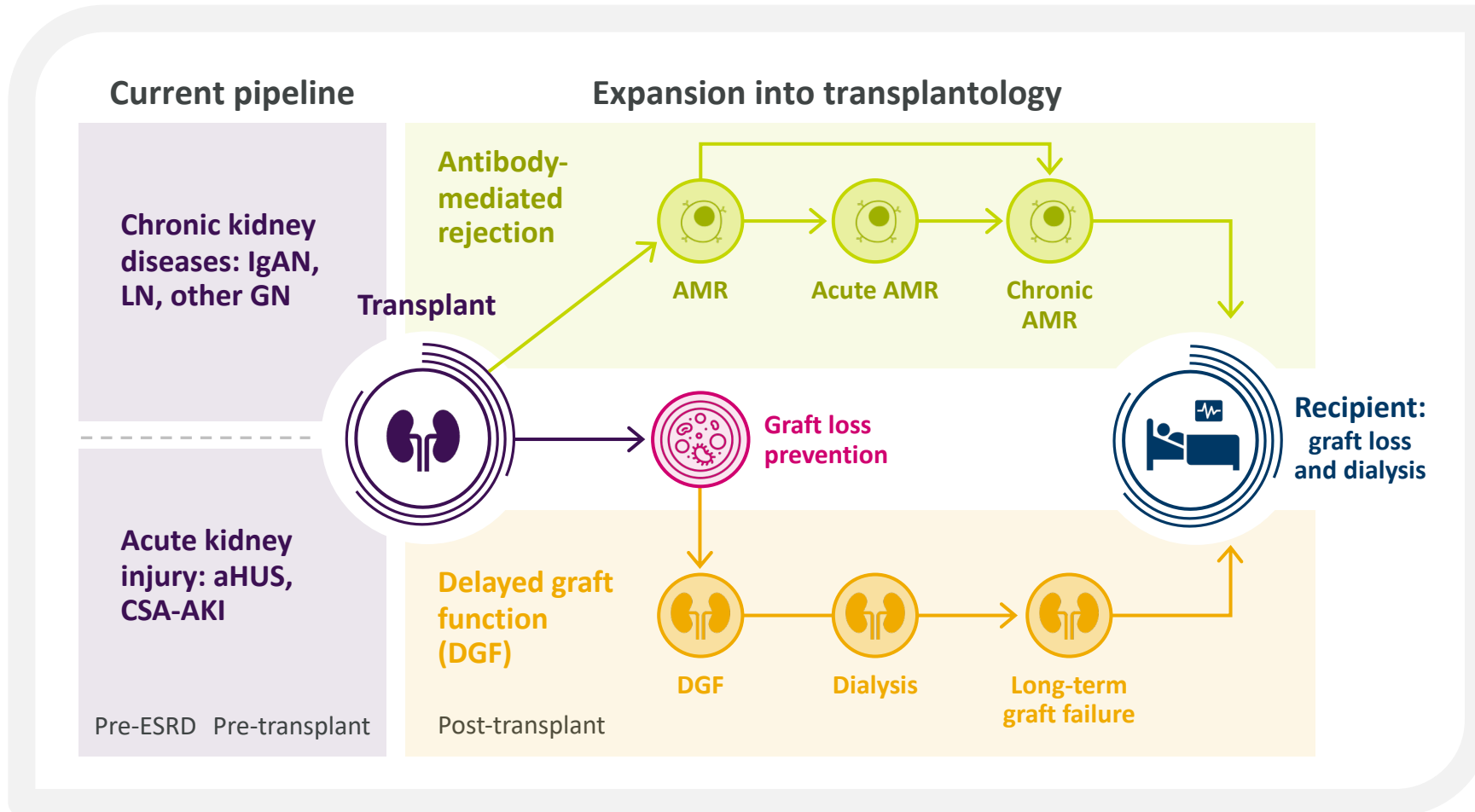
### Primary endpoints:

UPCR change at 34 weeks

eGFR evaluated at 106 weeks

Rapid, complete and sustained complement inhibition at Week 4

# Future plans to improve success rates of kidney transplant



**12% incidence of cAMR** in adult kidney transplants<sup>1</sup>

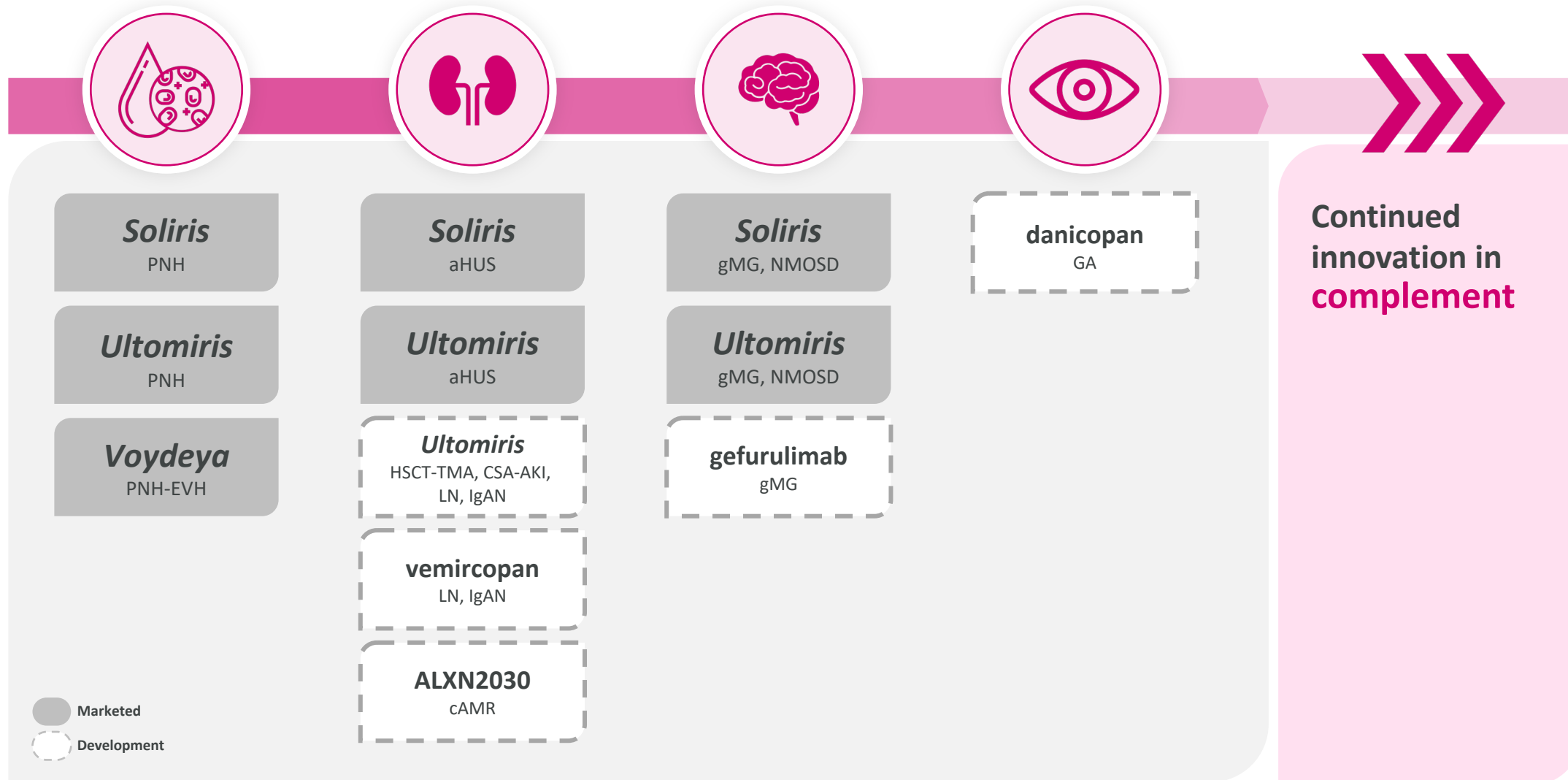
**75% of kidney transplant patients** diagnosed with cAMR lose their graft within 5 years<sup>2</sup>


**350k patients globally** waiting for kidney transplant<sup>3</sup>

**28% of donated kidneys** are discarded by surgeons in the US<sup>4</sup>

1. Carrie A Schinstock et al. Kidney Transplant with Low Levels of DSA or Low Positive B-Flow Crossmatch: An Underappreciated Option for Highly-Sensitized Transplant Candidates Transplantation. 2017 October ; 101(10): 2429–2439.  
 2. Betjes MGH, Roelen DL, van Agteren M, Kal-van Gestel J. Causes of Kidney Graft Failure in a Cohort of Recipients With a Very Long-Time Follow-Up After Transplantation. Front Med (Lausanne). 2022 Jun 6;9:842419. 3. [Newsletter Transplant - European Directorate for the Quality of Medicines & HealthCare \(edqm.eu\)](#). 4. [The Kidney Transplant Waitlist – What You Need to Know | National Kidney Foundation](#). Acronym definitions can be found in Glossary.

# Future innovation planned in complement



  
**Continued  
innovation in  
complement**

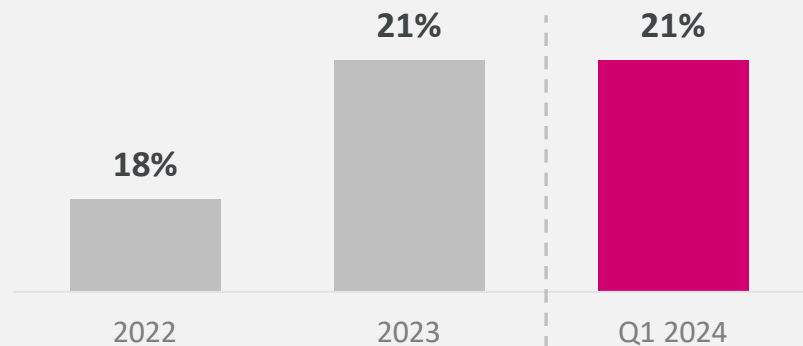




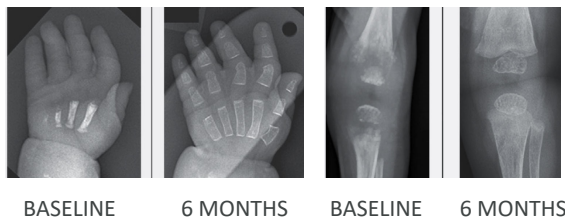
# Beyond Complement: bone and endocrine disease

# Hypophosphatasia – building on *Strensiq*, efzimfotase alfa drives innovation and expanded access

## *Strensiq* Total Revenue growth (%)



Radiographic changes from baseline in *Strensiq* patients



## efzimfotase alfa (ALXN1850)



**Convenient dosing**  
Q2W vs. 6–12 injections per two weeks with *Strensiq*



**Improved manufacturing**



**6x addressable patient population<sup>1</sup>**

**Phase III trials ongoing**  
with data anticipated >2025

- **MULBERRY**  
paediatric naïve
- **CHESTNUT**  
paediatric switch
- **HICKORY**  
adolescent/adult naïve

**Patient-centred innovation expands label to all HPP patients**

\*Peak Year Revenue, non-risk adjusted. FY 2022 growth rates on medicines acquired with Alexion have been calculated on a pro forma basis comparing to the corresponding period in the prior year. The growth rate shown has been calculated as though these changes had been implemented in FY 2021. All growth rates at CER. 1. 11.5K diagnosed patients in top 8 countries (US, EU5 (Germany, France, UK, Italy and Spain), Japan and China). 2. Vs. *Strensiq*, increase in addressable population driven by expanded indication of efzimfotase alfa to include adult patients with HPP, irrespective of clinical manifestation. Acronym definitions can be found in Glossary.

# Hypoparathyroidism – Amolyt Pharma acquisition yields potential best-in-class therapy with eneboparatide

\$1–3bn\*

## Clinical priorities

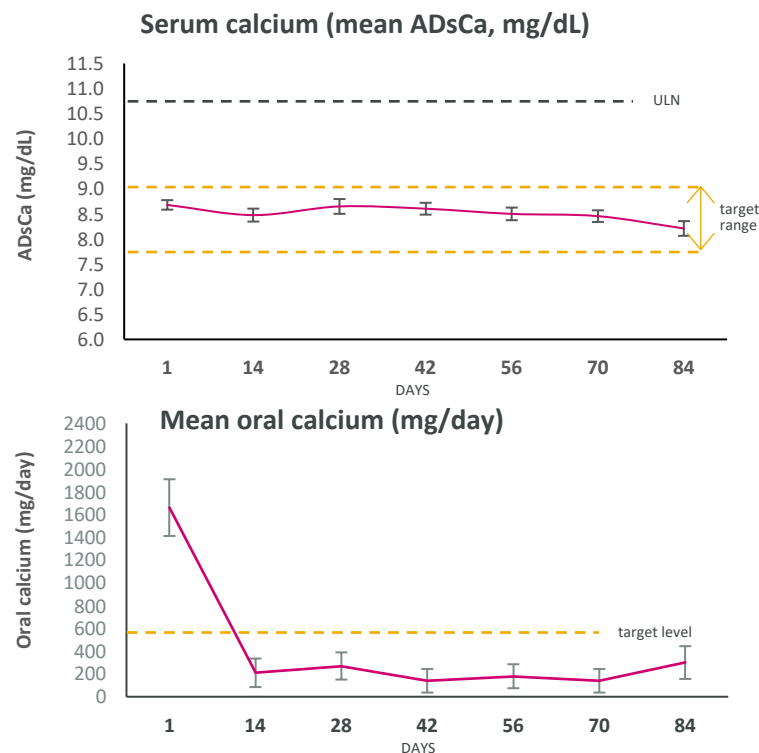
**Normalising serum calcium levels** preventing muscular cramps and cardiac effects

**Decreasing urinary calcium excretion** avoids loss of kidney function

**Preserving bone mineral density** preventing osteoporosis

**>250K patients in Top 8 countries<sup>1</sup>**  
**>50% are peri- or post-menopausal women**

## eneboparatide Phase IIa<sup>2</sup>



**Strengthening Alexion presence** in rare endocrinology

**Expansion** from *Strensiq* physician call points

**Phase III CALYPSO** data anticipated in 2025

\*Peak Year Revenue, non-risk adjusted. 1. Top 8: US, EU5 (Germany, France, UK, Italy and Spain), Japan and China. 2. An Open-label Phase 2 Study of eneboparatide, a Novel PTH Receptor 1 Agonist, in Hypoparathyroidism. The Journal of Clinical Endocrinology & Metabolism, dgae121.06 March 2024. Amolyt acquisition remains subject to customary external clearances; all clinical development plans mentioned herein subject to deal closure. Acronym definitions can be found in Glossary.



# Beyond Complement: amyloidosis

# Novel anti-fibril depletor mechanisms with potential to restore normal organ function

**ALXN2220** | transthyretin (ATTR) amyloidosis

**anselamimab** | light-chain (AL) amyloidosis

**Selectively binds to**  
ATTR amyloid fibrils

**~114k diagnosed**  
in US and EU5

**Phase III**  
recently initiated



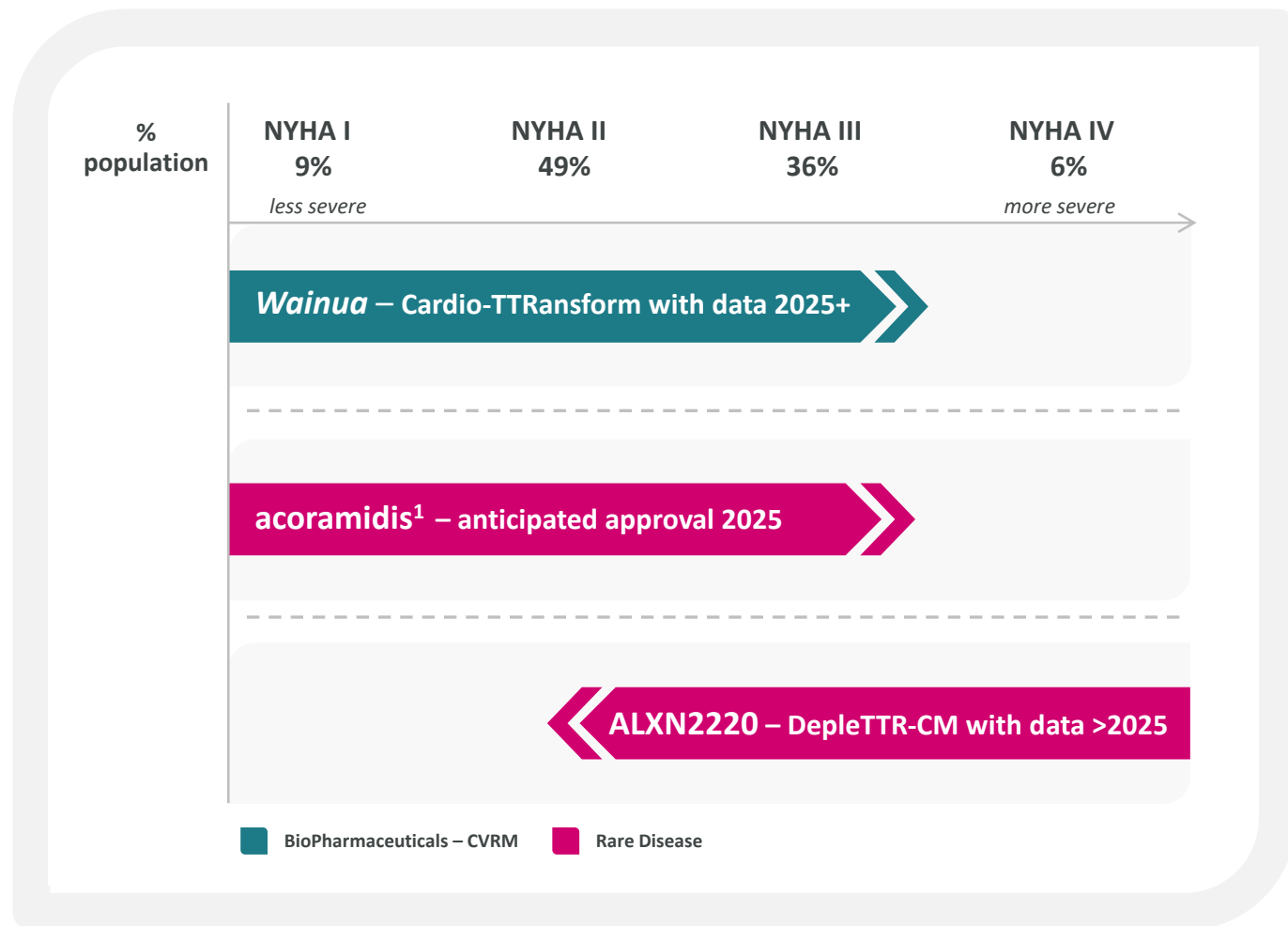
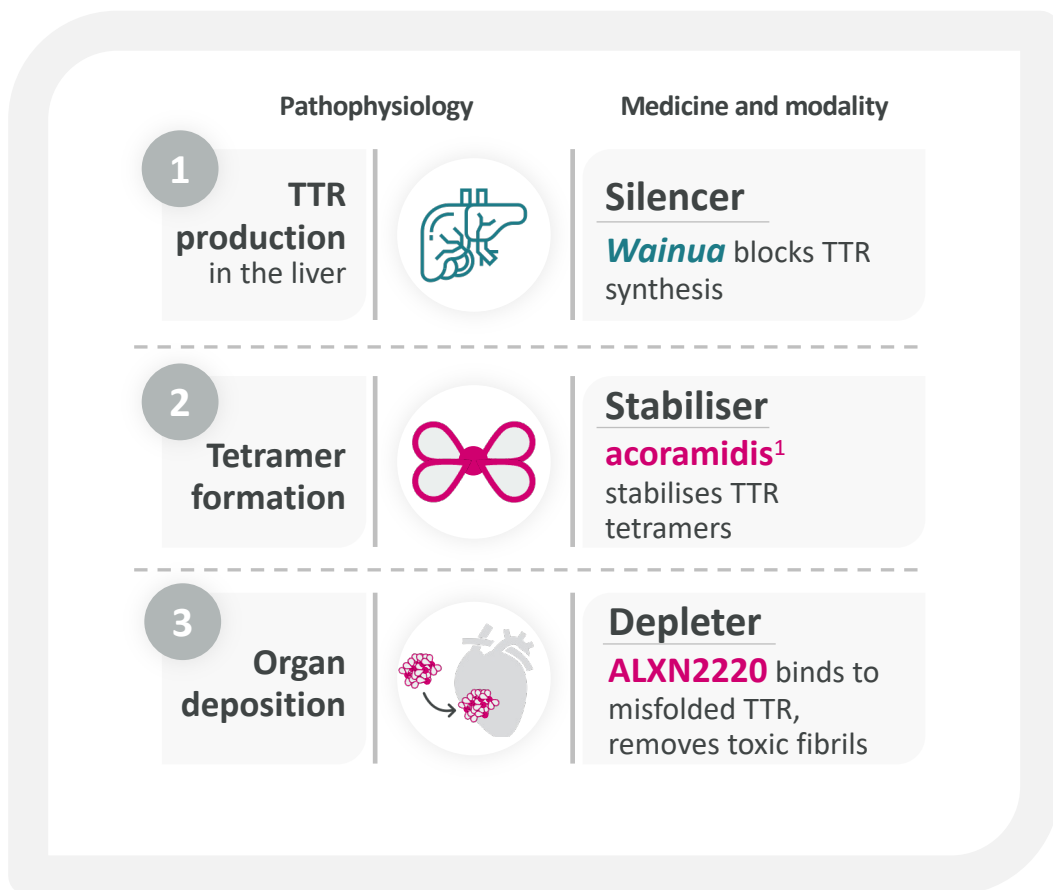
**Selectively binds**  
 $\kappa$  and  $\lambda$  light chain fibrils

**~28k diagnosed**  
in US and EU5

**Phase III enrollment**  
**complete** data in 2025

# Leveraging CVRM and Rare Disease expertise in ATTR-CM

## Complementary mechanisms

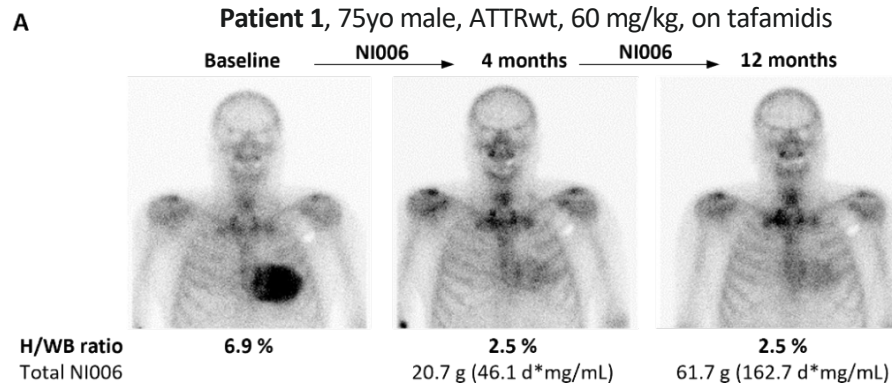


# Amyloidosis (ATTR-CM) – depleter mechanism with the potential to reverse course of disease

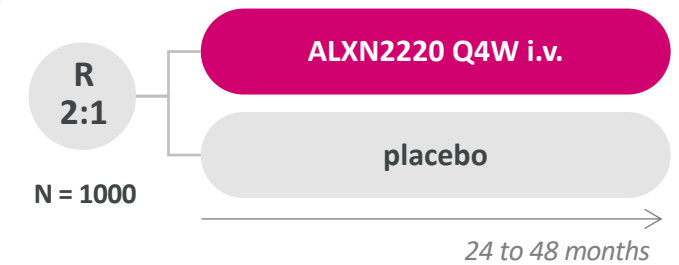
## ALXN2220 (NI006) Phase Ib<sup>1</sup>



## Phase III DepletTTR-CM



- ✓ Clearance of cardiac amyloid shown at 4 and 12 months
- ✓ Improvement in cardiac function (NT-proBNP)
- ✓ Potential monthly i.v. dosing



Primary endpoints include composite events


All-cause mortality

Cardiovascular events

**ALXN2220 selectively binds and removes misfolded amyloid fibrils to potentially improve overall survival**

\*Peak Year Revenue, non-risk adjusted. 1. Garcia-Pavia et al. NEJM Phase 1 Trial of Antibody ALXN2220 (NI006) for Depletion of Cardiac Transthyretin Amyloid. Representative images from serial bisphosphonate scintigraphy from one patient randomly assigned to receive ALXN2220 (NI006). Acronym definitions can be found in Glossary.

# Amyloidosis (AL) – transforming patient outcomes with potential first-in-class depleter

 \$1–3bn\*

## Cause

progressive accumulation of amyloid fibrils in tissues and organs including **kidneys and heart**

## Result

organ dysfunction and eventual death

**~9 months**

median overall survival for newly diagnosed Stage IIIb patients<sup>1</sup>

## anselamimab addressing key clinical outcomes

Novel depleter mechanism to eliminate deposited fibrils to improve



Overall survival



Cardiac function



Renal function

Two trials ongoing in Mayo Stage IIIa and IIIb

R  
2:1

anselamimab (+ anti-PCD) | i.v.

+ anti-PCD | i.v.

## Primary endpoints

- Time to all-cause mortality
- Number of cardiovascular hospitalisations

\*Peak Year Revenue, non-risk adjusted. 1. Gustine, Staron, Mendelson et. al. "Predictors of treatment response and survival outcomes in patients with advanced cardiac AL amyloidosis." October 2023.

Acronym definitions can be found in Glossary.





# Pipeline and new modalities

# Genomic medicines – unlocking transformative and potentially curative therapies for rare diseases



## Today

building capabilities through strategic investments

**80%**

of rare diseases are genetic<sup>1</sup>



## Tomorrow

engineering portfolio with curative potential

Advancing **up to 2 INDs** per year to 2030



## Future

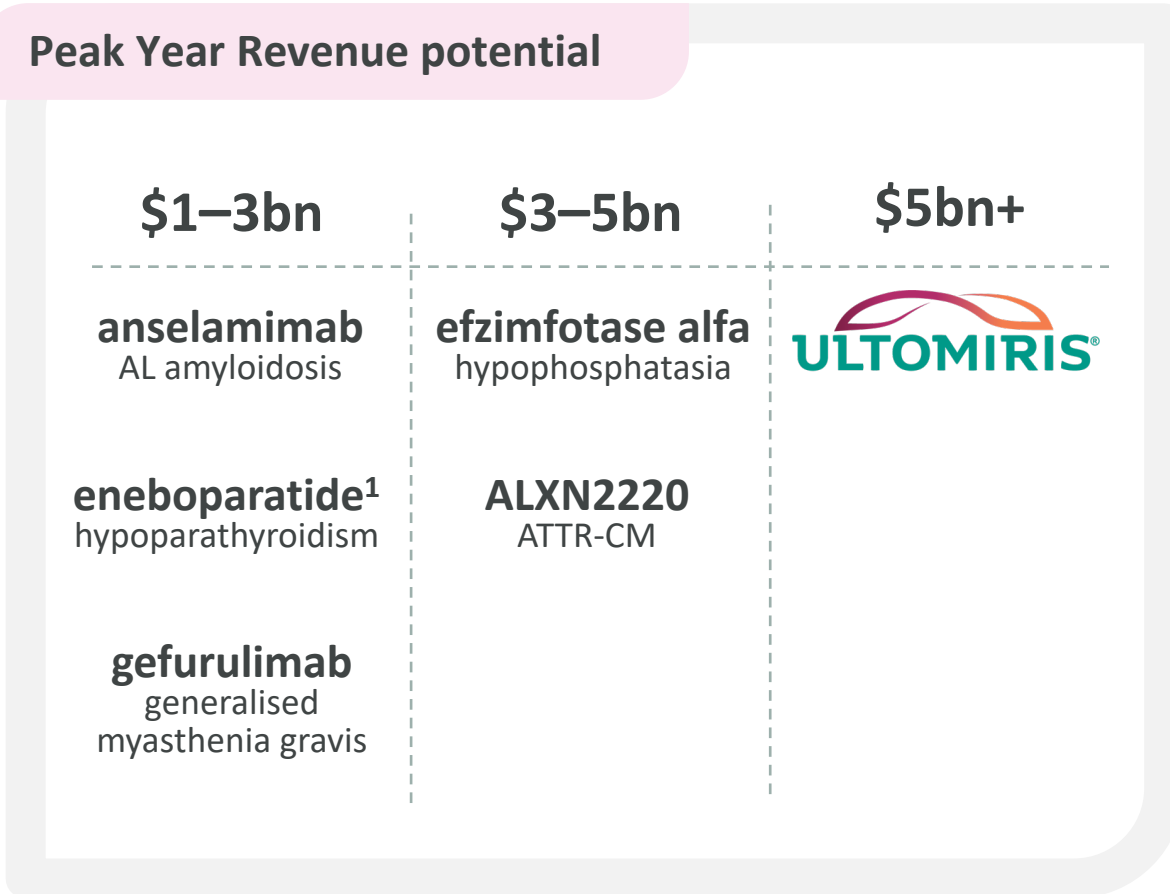
addressing diseases in areas of high unmet need

Revenue-generating potential **by 2030**

# Several major assets with blockbuster potential support our growth ambition

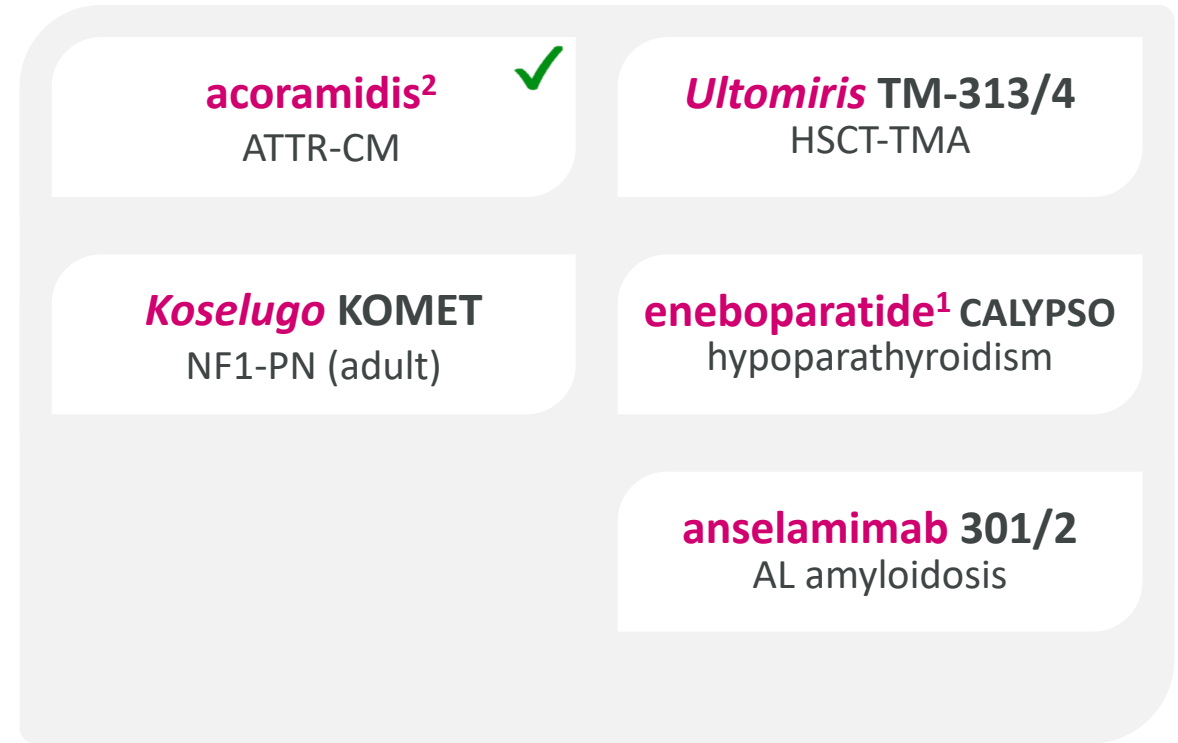
## Growth drivers to 2030 and beyond

### Peak Year Revenue potential



## 2024

## 2025



**Five Phase III readouts in 2024 and 2025**

1. Amlyt acquisition remains subject to customary external clearances; all clinical development plans mentioned herein subject to deal closure. 2. Alexion, AstraZeneca Rare Disease has rights to acoramidis in Japan. Peak Year Revenues could occur beyond 2030. Acronym definitions can be found in Glossary.

# Q&A session



**Marc Dunoyer**

CHIEF EXECUTIVE OFFICER,  
ALEXION



**Gianluca Pirozzi**

SVP, HEAD OF DEVELOPMENT,  
REGULATORY & SAFETY



**Nicola Heffron**

SVP, HEAD OF GLOBAL  
MARKETING & MARKET ACCESS



**Seng Cheng**

SVP, HEAD OF RESEARCH  
& PRODUCT DEVELOPMENT

# Appendix

# Glossary – 1 of 2

<b>1L, 2L, 3L</b>	first-, second-, third-line	<b>CLL</b>	chronic lymphocytic leukaemia	<b>GLP-1/ghu</b>	glucagon-like peptide 1 receptor/glucagon dual peptide agonist
<b>6MWT</b>	6-minute walk test	<b>cm</b>	centimetre	<b>GLP-1RA</b>	glucagon-like peptide 1 receptor agonist
<b>AAV</b>	adeno-associated virus	<b>CM</b>	cardiomyopathy	<b>gMG</b>	generalised myasthenia gravis
<b>ACE</b>	angiotensin-converting enzyme	<b>cMET</b>	c-mesenchymal epithelial transition factor	<b>GN</b>	glomerulonephritis
<b>AChR+</b>	acetylcholine receptor-positive	<b>COPD</b>	chronic obstructive pulmonary disease	<b>GPC3</b>	Glypican-3
<b>ADC</b>	antibody conjugate	<b>CRwNP</b>	chronic rhinosinusitis with nasal polyps	<b>GPRC5D</b>	G protein-coupled receptor class C group 5 member D
<b>ADsCa</b>	albumin-adjusted serum calcium	<b>CSA-AKI</b>	cardiac surgery-associated acute kidney injury	<b>GU</b>	genitourinary
<b>AER</b>	annual exacerbation rate	<b>ctDNA</b>	circulating tumour DNA	<b>GYN</b>	gynaecologic
<b>AEs</b>	adverse effects	<b>CTLA4</b>	cytotoxic T-lymphocyte associated protein 4	<b>HbA1c</b>	glycated haemoglobin
<b>AGA</b>	actional genomic alteration	<b>CTx</b>	chemotherapy	<b>HCC</b>	hepatocellular carcinoma
<b>aHUS</b>	atypical haemolytic uraemic syndrome	<b>CV</b>	cardiovascular	<b>HER2</b>	human epidermal growth factor receptor 2
<b>AL amyloidosis</b>	light-chain amyloidosis	<b>CVRM</b>	Cardiovascular, Renal and Metabolism	<b>HF</b>	heart failure
<b>AML</b>	acute myelogenous leukaemia	<b>DDR</b>	DNA damage response	<b>HFrEF</b>	heart failure with reduced ejection fraction
<b>AMR</b>	antibody mediated rejection	<b>DGF</b>	delayed graft function	<b>HK</b>	hyperkalaemia
<b>anti-PCD</b>	anti plasma cell dyscrasia	<b>DLBCL</b>	diffuse large B-cell lymphoma	<b>HLR</b>	high-level results
<b>AQP4+</b>	aquaporin-4 antibody positive	<b>dnTGFb</b>	dominant-negative transforming growth factor-beta	<b>hMPV</b>	human metapneumovirus
<b>ARB</b>	angiotensin receptor blockers	<b>dPTEN</b>	phosphatase and tensin homolog deficient	<b>HNSCC</b>	head and neck squamous cell carcinoma
<b>ASCO</b>	American Society of Clinical Oncology	<b>EBITDA</b>	Earnings before interest, tax, depreciation and amortisation	<b>HR</b>	hazard ratio
<b>ASI</b>	aldosterone synthase inhibitor	<b>EGFR</b>	epidermal growth factor receptor	<b>HR+</b>	hormone receptor positive
<b>ASO</b>	antisense oligonucleotide	<b>eGFR</b>	estimated glomerular filtration rate	<b>HRR</b>	homologous recombination repair
<b>ATTR-CM</b>	transthyretin amyloid cardiomyopathy	<b>EGPA</b>	eosinophilic granulomatosis with polyangiitis	<b>HSCT-TMA</b>	hematopoietic stem cell transplantation-associated thrombotic microangiopathy
<b>ATTR-PN</b>	transthyretin amyloid polyneuropathy	<b>EM</b>	Emerging Markets	<b>i.v.</b>	intravenous
<b>B-ALL</b>	B-cell acute lymphoblastic leukaemia	<b>EOS</b>	eosinophil	<b>IBD</b>	inflammatory bowel disease
<b>BCMA</b>	B-cell maturation antigen	<b>EPI</b>	epigenetics	<b>ICS</b>	inhaled corticosteroid
<b>BRCA</b>	breast cancer gene	<b>EPS</b>	earnings per share	<b>ICU</b>	intensive care unit
<b>BTC</b>	biliary tract cancer	<b>ERoW</b>	Established Rest of World	<b>IgAN</b>	IgA nephropathy
<b>BTKi</b>	Bruton's tyrosine kinase	<b>ESR1</b>	estrogen receptor alpha	<b>IIT</b>	investigated initiated trial
<b>C5</b>	complement component 5	<b>ESRD</b>	end stage renal disease	<b>iJAK1</b>	inhaled Janus kinase
<b>CAGR</b>	compound adjusted growth rate	<b>ETA RA</b>	endothelin receptor A antagonist	<b>IL-33</b>	interleukin-33
<b>cAMR</b>	chronic antibody-mediated rejection	<b>ETARA</b>	endothelin receptor A antagonist	<b>IL-5</b>	interleukin-5
<b>CAR-T</b>	chimeric antigen receptor T-cells	<b>FDC</b>	fixed dose combination	<b>IND</b>	investigational new drug
<b>CD19</b>	Cluster of differentiation 19	<b>FeNO</b>	fractional exhaled nitric oxide	<b>IO</b>	Immuno-oncology
<b>CD3</b>	Cluster of differentiation 3	<b>FL</b>	Follicular lymphoma	<b>IPF</b>	idiopathic pulmonary fibrosis
<b>CDK4/6i</b>	cyclin-dependent kinase 4/6 inhibitor	<b>FLAP</b>	5-lipoxygenase activating protein	<b>IRA</b>	Inflation Reduction Act
<b>CER</b>	constant exchange rates	<b>FRα</b>	folate receptor alpha	<b>iTSLP</b>	inhaled thymic stromal lymphopoietin
<b>CI</b>	confidence interval	<b>FX</b>	foreign exchange	<b>ITT</b>	intent to treat
<b>CKD</b>	chronic kidney disease	<b>G7</b>	US, Japan, EU5	<b>IVIg</b>	intravenous immunoglobulin
<b>CLDN 18.2</b>	Claudin-18.2	<b>GA</b>	geographic atrophy		

# Glossary – 2 of 2

<b>K+</b>	potassium	<b>NST</b>	neoadjuvant systemic treatment	<b>RSV</b>	respiratory syncytial virus
<b>KCCQ</b>	Kansas City Cardiomyopathy Questionnaire	<b>NT-proBNP</b>	N-terminal pro-B-type natriuretic peptide	<b>s. asthma</b>	severe asthma
<b>LA amylin</b>	long-acting amylin	<b>NYHA</b>	New York Heart Association	<b>s.c.</b>	subcutaneous
<b>LABA</b>	long-acting beta 2-agonists	<b>oGLP1</b>	oral glucagon-like receptor peptide 1	<b>SABA</b>	short acting beta agonist
<b>LAMA</b>	long-acting muscarinic antagonists	<b>oPCSK9</b>	oral protein convertase subtilisin/kexin type 9	<b>SBP</b>	systolic blood pressure
<b>LCM</b>	life cycle management	<b>ORR</b>	overall response rate	<b>SBRT</b>	stereotactic brain radiotherapy
<b>LDL-C</b>	low-density lipoprotein cholesterol	<b>oRXFP1</b>	oral relaxin family peptide receptor 1	<b>SC</b>	subcutaneous
<b>LN</b>	lupus nephritis	<b>OS</b>	overall survival	<b>SG&amp;A</b>	Selling, General and Administrative
<b>LoE</b>	loss of exclusivity	<b>PALB2m</b>	partner and localizer of BRCA2	<b>SGLT2i</b>	sodium/glucose cotransporter 2 inhibitor
<b>LS-SCLC</b>	limited stage small-cell lung cancer	<b>PARP1</b>	poly(ADP-ribose) polymerase-1	<b>sK</b>	serum potassium
<b>LV</b>	left ventricular	<b>PARPi</b>	poly-ADP ribose polymerase inhibitor	<b>SLE</b>	systemic lupus erythematosus
<b>mAb</b>	monoclonal antibody	<b>PD1</b>	programmed cell death protein 1	<b>SoC</b>	standard of care
<b>MASH</b>	metabolic dysfunction-associated steatohepatitis, also known as non-alcoholic steatohepatitis (NASH)	<b>PD-L1</b>	programmed cell death ligand 1	<b>ST2</b>	suppression of tumorigenicity 2
<b>MASLD</b>	metabolic dysfunction-associated steatotic liver disease	<b>PFS</b>	progression free survival	<b>Stg. I/II/III</b>	Stage I/II/III
<b>mBC</b>	metastatic breast cancer	<b>PIK3CA</b>	phosphatidylinositol-4,5-biphosphate 3-kinase catalytic subunit	<b>Stg. III u/r NSCLC</b>	Stage III unresectable non-small cell lung cancer
<b>MCL</b>	mantle cell lymphoma	<b>PK/PD</b>	pharmacokinetic/pharmacodynamic	<b>T2D</b>	type-2 diabetes
<b>mDOR</b>	median duration of response	<b>PLEX</b>	plasma exchange	<b>T8</b>	US, China, Japan, EU5
<b>mg/dL</b>	milligrams per decilitre	<b>PN</b>	polyneuropathy	<b>TCE</b>	T-cell engager
<b>MGFA</b>	Myasthenia Gravis Foundation of America	<b>PNH</b>	paroxysmal nocturnal haemoglobinuria	<b>tCO2e</b>	tonnes of carbon dioxide equivalent
<b>mHSPC</b>	metastatic hormone sensitive prostate cancer	<b>PNH-EVH</b>	paroxysmal nocturnal haemoglobinuria with extravascular haemolysis	<b>TCR</b>	T-cell receptor
<b>mL</b>	millilitre	<b>PNPLA3</b>	phospholipase domain-containing protein 3	<b>TDR</b>	tumour drivers and resistance
<b>MM</b>	multiple myeloma	<b>PP</b>	plasmapheresis	<b>TIGIT</b>	T-cell immunoreceptor with immunoglobulin and ITIM domains
<b>MoA</b>	mechanism of action	<b>PSA</b>	prostate-specific antigen	<b>TIM-3</b>	T-cell immunoglobulin and mucin domain-containing protein
<b>MPO</b>	myeloperoxidase	<b>PSA50</b>	prostate-specific antigen 50	<b>TKI</b>	tyrosine kinase inhibitor
<b>MRA</b>	mineralocorticoid receptor antagonist	<b>PTEN</b>	phosphatase and TENsin homolog deleted on chromosome 10	<b>TNBC</b>	triple negative breast cancer
<b>MRM</b>	mineralocorticoid receptor modulator	<b>PYR</b>	peak year revenue	<b>TP53</b>	tumour protein 53
<b>n/m</b>	not material	<b>Q2W</b>	every 2 weeks	<b>Treg</b>	Regulatory T-cell
<b>NBRx</b>	new-to-brand prescription	<b>Q4W</b>	every 4 weeks	<b>TROP2</b>	trophoblast cell surface antigen 2
<b>Neo-adj</b>	neoadjuvant	<b>Q8W</b>	every 8 weeks	<b>TTR</b>	transthyretin
<b>NF1-PN</b>	neurofibromatosis type 1-plexiform neurofibromas	<b>QCS</b>	quantitative continuous scoring	<b>u/r HTN</b>	uncontrolled or treatment resistant hypertension
<b>ngSERD</b>	next-generation oral selective estrogen receptor degrader	<b>QoQ</b>	quarter on quarter	<b>UACR</b>	urinary albumin/creatinine ratio
<b>NHA</b>	novel hormone agent	<b>R&amp;D</b>	research and development	<b>ULN</b>	upper limit of normal
<b>NME</b>	new molecular entity	<b>R&amp;I</b>	Respiratory and Immunology	<b>V&amp;I</b>	Vaccines and Immune Therapies
<b>NMOSD</b>	neuromyelitis optica spectrum disorder	<b>r/r</b>	relapsed/refractory	<b>VLP</b>	virus-like particle
<b>NP</b>	nasal polyps	<b>RA</b>	rheumatoid arthritis		
<b>NRDL</b>	national reimbursement drug list	<b>RAGE</b>	receptor for advanced glycation end products		
<b>NSCLC</b>	non-small cell lung cancer	<b>RC</b>	radioconjugates		
		<b>RP2D</b>	recommended Phase II dose		