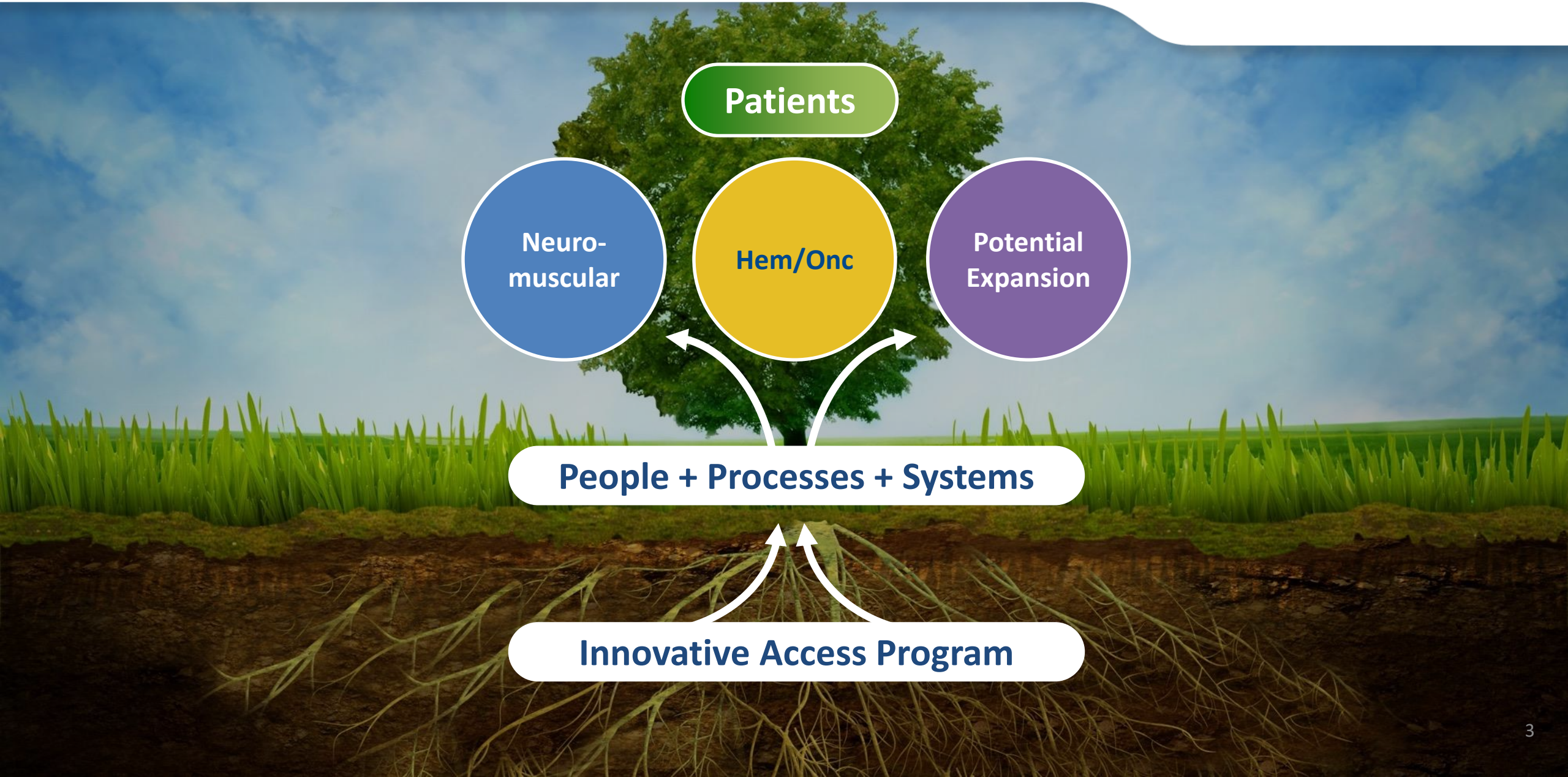

# Wedbush Healthcare Conference
# August 2019

# Forward-Looking Statements

THIS PRESENTATION HAS BEEN PREPARED BY ARGENX SE ("ARGENX" OR THE "COMPANY") FOR INFORMATIONAL PURPOSES ONLY AND NOT FOR ANY OTHER PURPOSE. NOTHING CONTAINED IN THIS PRESENTATION IS, OR SHOULD BE CONSTRUED AS, A RECOMMENDATION, PROMISE OR REPRESENTATION BY THE PRESENTER OR THE COMPANY OR ANY DIRECTOR, EMPLOYEE, AGENT, OR ADVISER OF THE COMPANY. THIS PRESENTATION DOES NOT PURPORT TO BE ALL-INCLUSIVE OR TO CONTAIN ALL OF THE INFORMATION YOU MAY DESIRE. THIS PRESENTATION ALSO CONTAINS ESTIMATES AND OTHER STATISTICAL DATA MADE BY INDEPENDENT PARTIES AND BY US RELATING TO MARKET SIZE AND GROWTH AND OTHER DATA ABOUT OUR INDUSTRY. THIS DATA INVOLVES A NUMBER OF ASSUMPTIONS AND LIMITATIONS, AND YOU ARE CAUTIONED NOT TO GIVE UNDUE WEIGHT TO SUCH ESTIMATES.

Safe Harbor: Certain statements contained in this presentation, other than present and historical facts and conditions independently verifiable at the date hereof, may constitute forward-looking statements. Examples of such forward-looking statements include those regarding our investigational product candidates and preclinical studies and clinical trials, and the status, plans, timing of expected data readouts and related presentations and related results thereof, including the design of our trials and the availability of data from them, the timing and achievement of our product candidate development activities, future results of operations and financial positions, including potential milestones, business strategy, plans and our objectives for future operations. When used in this presentation, the words "anticipate," "believe," "can," "could," "estimate," "expect," "intend," "is designed to," "may," "might," "will," "plan," "potential," "predict," "objective," "should," or the negative of these and similar expressions identify forward-looking statements. Such statements, based as they are on the current analysis and expectations of management, inherently involve numerous risks and uncertainties, known and unknown, many of which are beyond the Company's control. Such risks include, but are not limited to: the impact of general economic conditions, general conditions in the biopharmaceutical industries, changes in the global and regional regulatory environments in the jurisdictions in which the Company does or plans to do business, market volatility, fluctuations in costs and changes to the competitive environment. Consequently, actual future results may differ materially from the anticipated results expressed in the forward-looking statements. In the case of forward-looking statements regarding investigational product candidates and continuing further development efforts, specific risks which could cause actual results to differ materially from the Company's current analysis and expectations include: failure to demonstrate the safety, tolerability and efficacy of our product candidates; final and quality controlled verification of data and the related analyses; the expense and uncertainty of obtaining regulatory approval, including from the U.S. Food and Drug Administration and European Medicines Agency; the possibility of having to conduct additional clinical trials; our ability to obtain and maintain intellectual property protection for our product candidates; and our reliance on third parties such as our licensors and collaboration partners regarding our suite of technologies and product candidates. Further, even if regulatory approval is obtained, biopharmaceutical products are generally subject to stringent on-going governmental regulation, challenges in gaining market acceptance and competition. These statements are also subject to a number of material risks and uncertainties that are described in the Company's filings with the U.S. Securities and Exchange Commission ("SEC"), including in argenx's most recent annual report on Form 20-F filed with the SEC as well as subsequent filings and reports filed by argenx with the SEC. The reader should not place undue reliance on any forward-looking statements included in this presentation. These statements speak only as of the date made and the Company is under no obligation and disavows any obligation to update or revise such statements as a result of any event, circumstances or otherwise, unless required by applicable legislation.

# argenx 2021:
# Becoming a Global Integrated Immunology Biotech

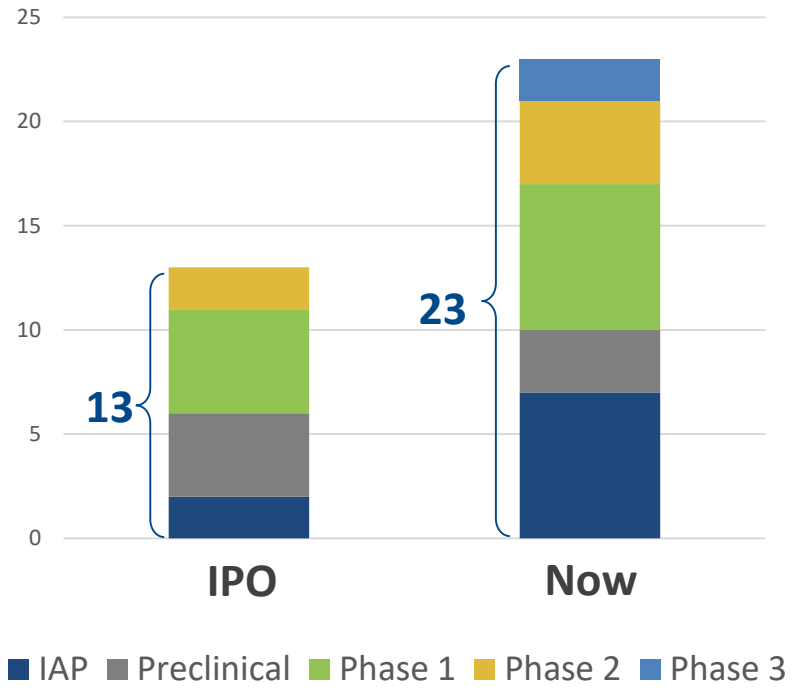


# Impressive Value Creation Since IPO

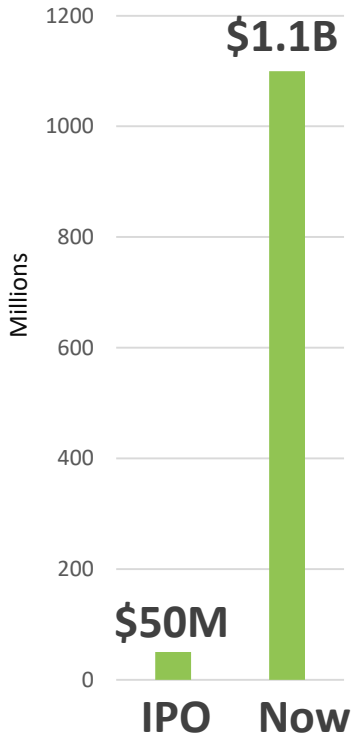
## Accelerating & expanding development programs

Key Data

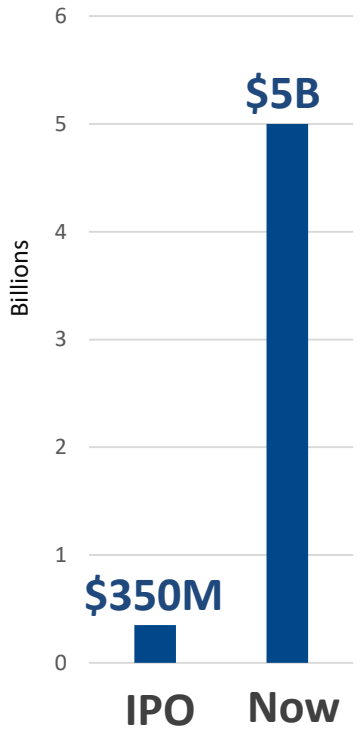
MG & ITP | Interim PV | AML




## Well-capitalized to advance to the next level

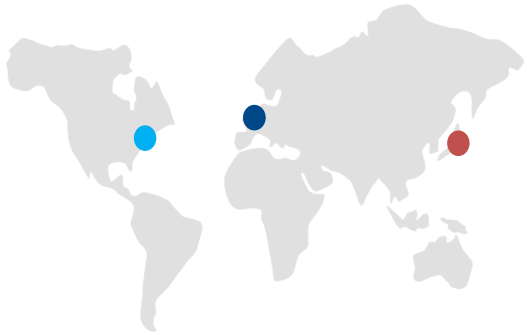



Cash




Market Cap

## Global expansion



- Ghent
- Boston (2018)
- Tokyo (2019)

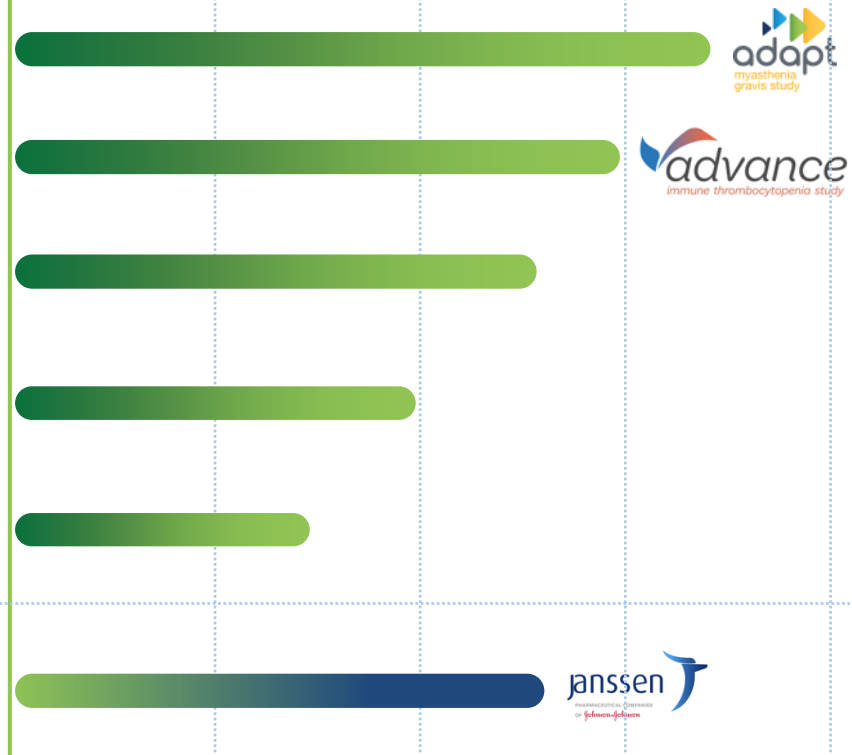
# argenx Today:
# Building Leadership in Immunology

| | |
|---|---|
| Late-stage immunology company | Two Phase 3 trials in progress by end of 2019 |
| Wholly-owned pipeline-in-a-product assets | Proof-of-concept in two beachhead indications |
| Validating oncology collaborations | Maintained 50% of cusatuzumab commercial rights |
| Innovative Access Program | One new asset per year to grow pipeline |
| Well-funded with cash into 2021 | $1.05B in cash to execute on ambitious plan |

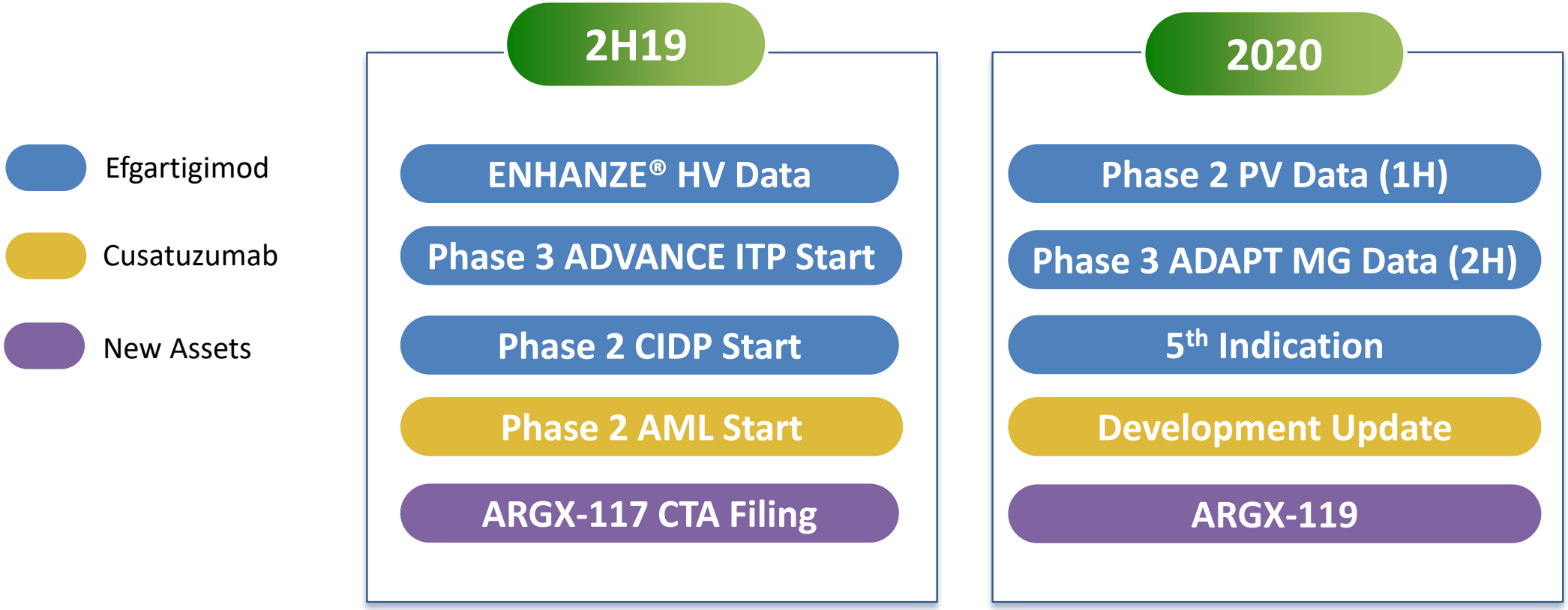
# Deep Proprietary Pipeline of Highly Differentiated Product Candidates

## Targeting high-value rapid-growth markets

| Product Candidate | Target | Indication | Next milestones |
|---|---|---|---|
| ARGX-113 Efgartigimod | FcRn | Myasthenia Gravis (MG) | Results 2H20 |
| | | Immune Thrombocytopenia (ITP) | Ph3 IV trial start 2H19 |
| | | Pemphigus Vulgaris (PV) | Topline results 1H20 |
| | | Chronic Inflammatory Demyelinating Polyneuropathy (CIDP) | Ph2 trial start 2H19 |
| | | ENHANZE® SC | Results YE19 |
| ARGX-110 Cusatuzumab | CD70 | Acute Myeloid Leukemia (AML) | Ph2 registration-directed trial start 2H19 |
| ARGX-117 | C2 | Severe Autoimmunity IV/ENHANZE® SC | CTA filing YE19 |
| ARGX-118 | Galectin-10 | Airway Inflammation | Lead selected |



# Multiple Value-Creating Milestones Through 2020




**Well-capitalized to execute on ambitious development plan into 2021**

# Innovative Access Program

Unique discovery engine to identify novel target biology

## Accessing Novel Targets Through Collaboration

**argenx**

**Antibody Expertise**

SIMPLE Antibody™, NHance®, ABDEG™, POTELLIGENT®

**Top Academic Institutions & Biotechs**

**Disease Biology Expertise**

Texas A&M, Bern, Utrecht, Louvain, Penn, Columbia, Torino, de Duve, VIB

### Co-creating first-in-class assets

**WHOLLY-OWNED**

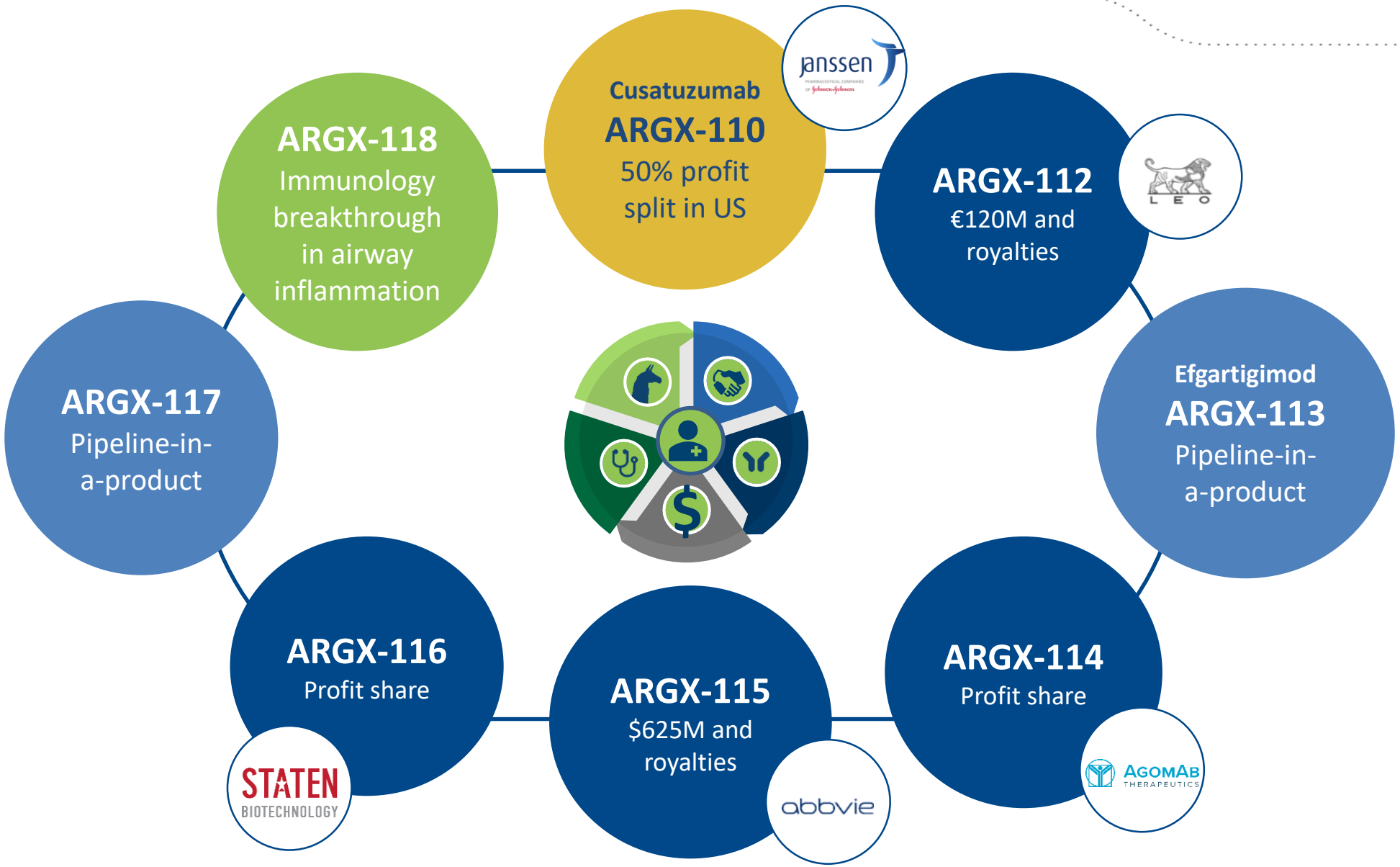
ARGX-113
ARGX-110
(Co-developed with Janssen)
ARGX-117
ARGX-118

**PARTNERED**

ARGX-115 abbvie
ARGX-112 LEO
ARGX-116 STATEN
ARGX-114 AGOMAB THERAPEUTICS

**5-10 ongoing programs at any given time**

# Serial Value Creation from Novel Targets

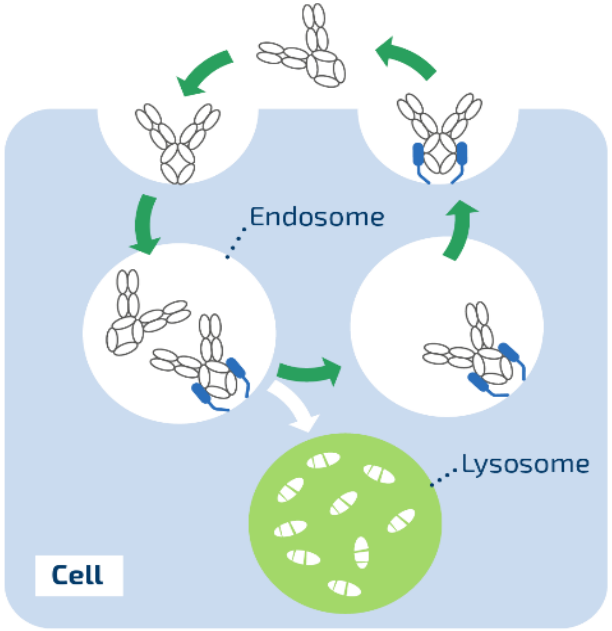


# Late-stage Development Product Candidates: Efgartigimod and Cusatuzumab

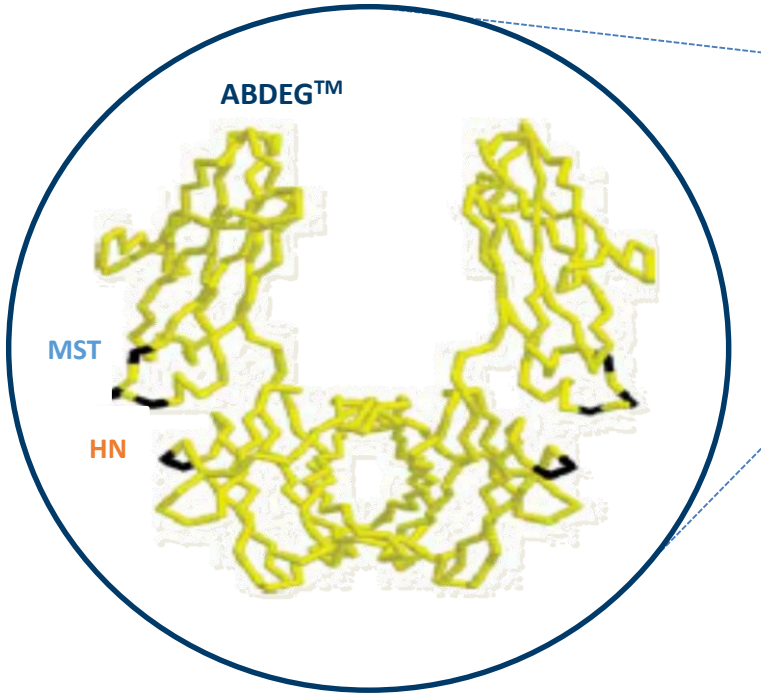
# Efgartigimod: Human IgG1 Fc Fragment with Proprietary ABDEG™ Mutations

Exploits natural Fc/FcRn interaction and retains pH dependent binding

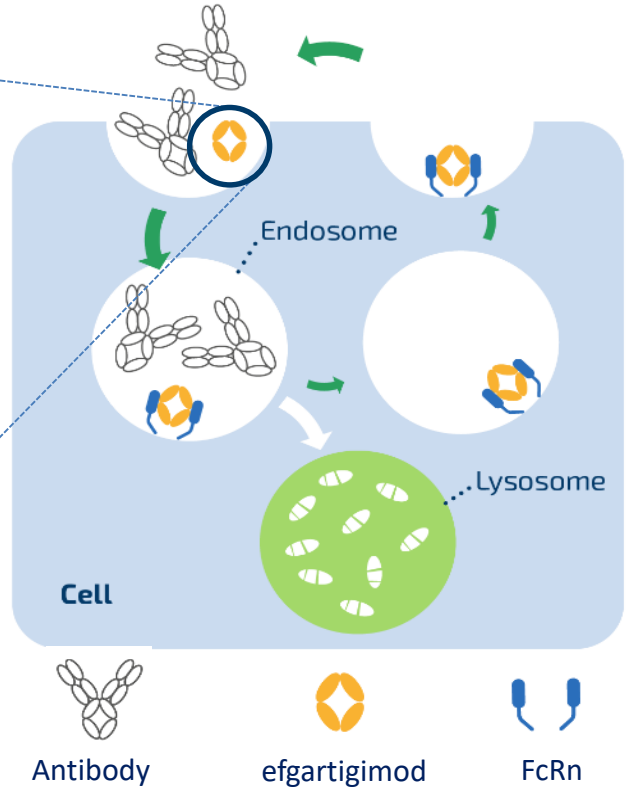
## IgG antibodies recycle through FcRn[1]…



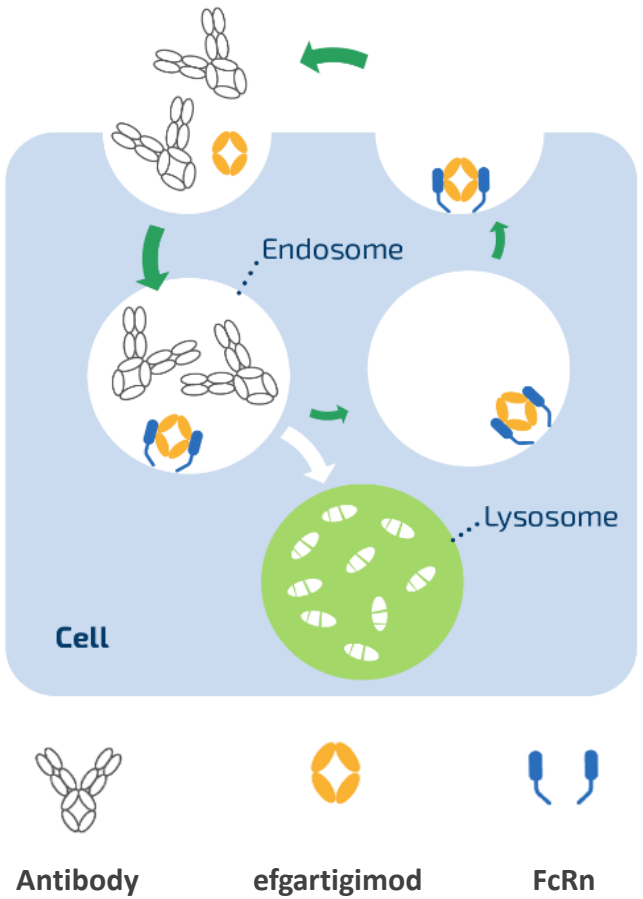

## efgartigimod potently blocks FcRn…




## leading to IgG elimination[2]



# Efgartigimod: Best-In-Class Potential With Broad Applicability




## Efficacy – Set the bar high in Phase 2 studies

75% of gMG patients achieved durable responses
~50% response rate in heavily pre-treated ITP patients

## Safety – No class effect

>150 patients treated
No safety signal detected (no trend in headaches or GI symptoms; no drop in albumin)

## Convenience – Optionality for patients

IV (10mg/kg): 60min infusion, no premedication, no infusion reactions
SC maintenance product (165mg/ml): 2ml push
SC ENHANZE® product through strategic collaboration with Halozyme

# Efgartigimod: Pipeline-in-a-Product Opportunity

Clinical proof-of-concept achieved for neuromuscular and hematology indications

## Landscape of IgG-mediated severe autoimmune diseases (sampling)

Immune Thrombocytopenia
Scleroderma
Lupus
Epidermolysis Bullosa Acquisita
Myasthenia Gravis
Rheumatoid Arthritis
Pemphigus
Multiple Sclerosis
Anca Vasculitis
Bullous Pemphigoid

**Solid Biology Rationale**
Disease proven to be predominantly mediated by pathogenic IgGs

**Feasible for Biotech**
Orphan potential, economically viable, efficient clinical & regulatory pathway

**Proof-of Concept:**

- Myasthenia Gravis ✓ → Neuromuscular Diseases
- Immune Thrombocytopenia ✓ → Hematology Disorders
- Pemphigus Vulgaris → Blistering Diseases

**Therapeutic Area Beachheads with Expansion Possibilities into Adjacent Indications**

Chronic Inflammatory Demyelinating Polyneuropathy (CIDP)

**Phase 2 CIDP study to start in 2H19**

# Efgartigimod in Myasthenia Gravis – Phase 3 ADAPT Trial Ongoing

## Enrollment on track – data expected 2H20

- Randomized, double-blind, placebo-controlled, multicenter trial enrolling 150 patients in North America, Europe and Japan
- Enrolling AChR positive and AChR negative patients with disease driven primarily by MuSK and LRP4 autoantibodies
- 10 mg/kg IV dose over 26-week period
- Patients eligible to roll over into 1-year open-label extension trial

adapt myasthenia gravis study

**Primary objective**

**MG Activities of Daily Living (MG-ADL) Score**

**Secondary objectives**

**Efficacy, safety, tolerability, QoL and impact on normal daily activities measures**

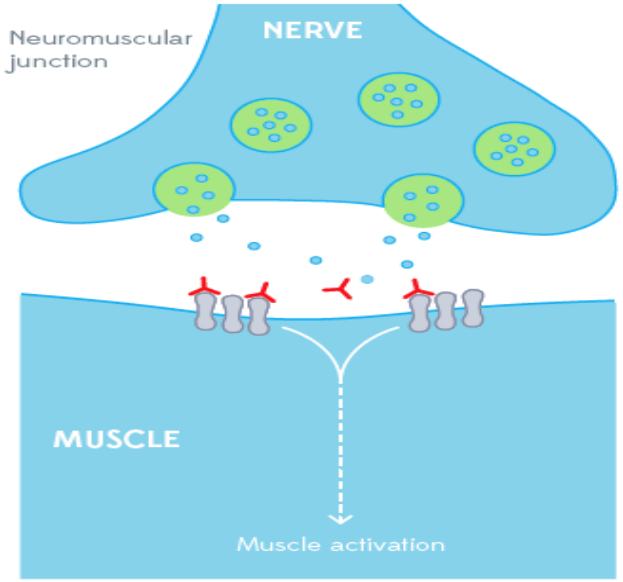
Neurology® Data from completed Phase 2 trial published in *Neurology* demonstrating that:

- Treatment with efgartigimod resulted in clinically meaningful and sustained improvement in disease scores, consistent across four MG scales
- Efgartigimod has a clean tolerability profile in line with HV study with no withdrawals or apparent differences between patients or placebo groups

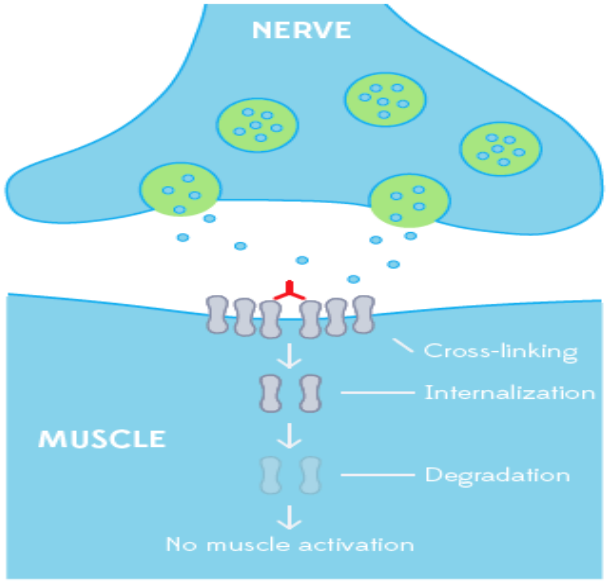
# Efgartigimod in Myasthenia Gravis

## Role of pathogenic autoantibodies very well-characterized

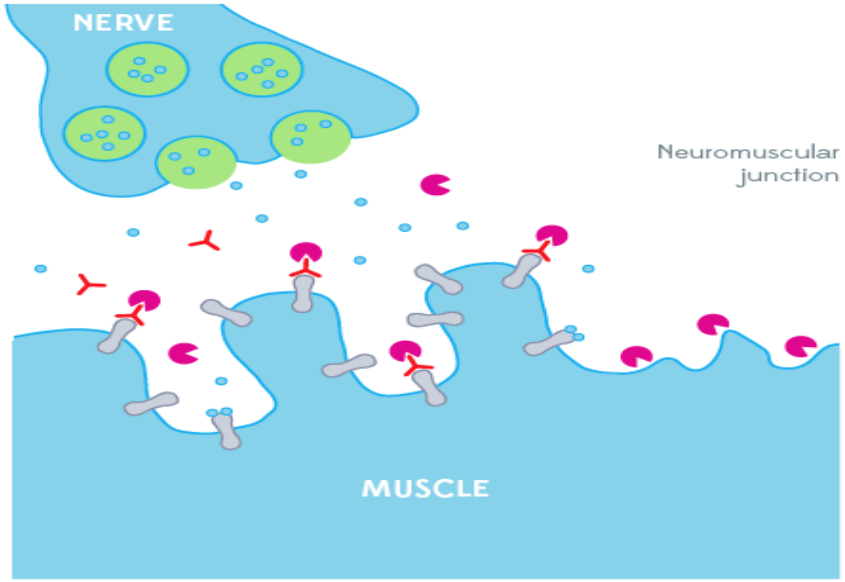
**Block acetylcholine from binding to AChR**




**Cross-link and internalize AChRs reducing number of binding sites**




**Recruit complement which can destroy muscle surface**

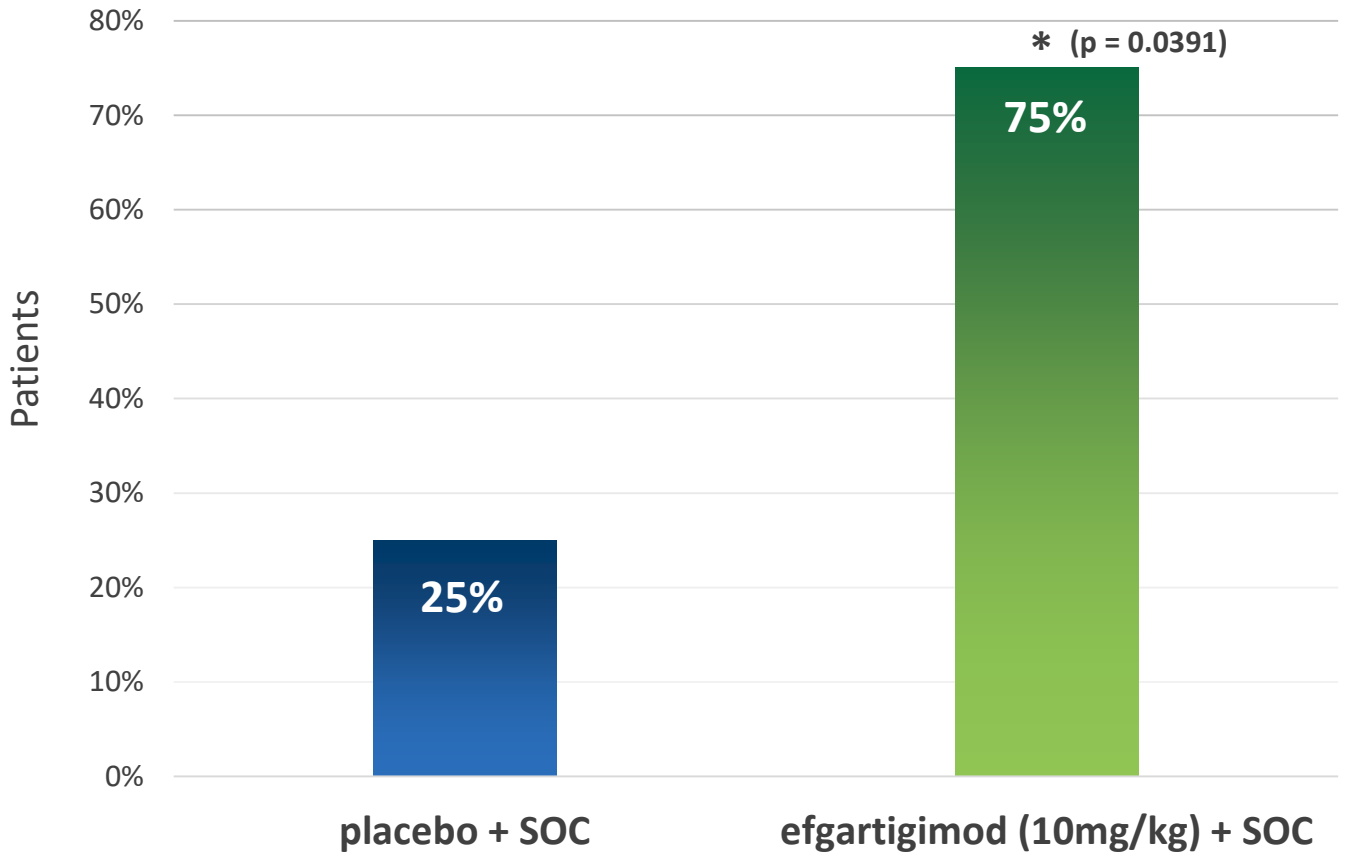



**IgG antibody** | **Acetylcholine receptor** | **Acetylcholine** | **Complement**

# Efgartigimod in Myasthenia Gravis – Strong Phase 2 Efficacy Results

75% of treated patients achieved lasting response

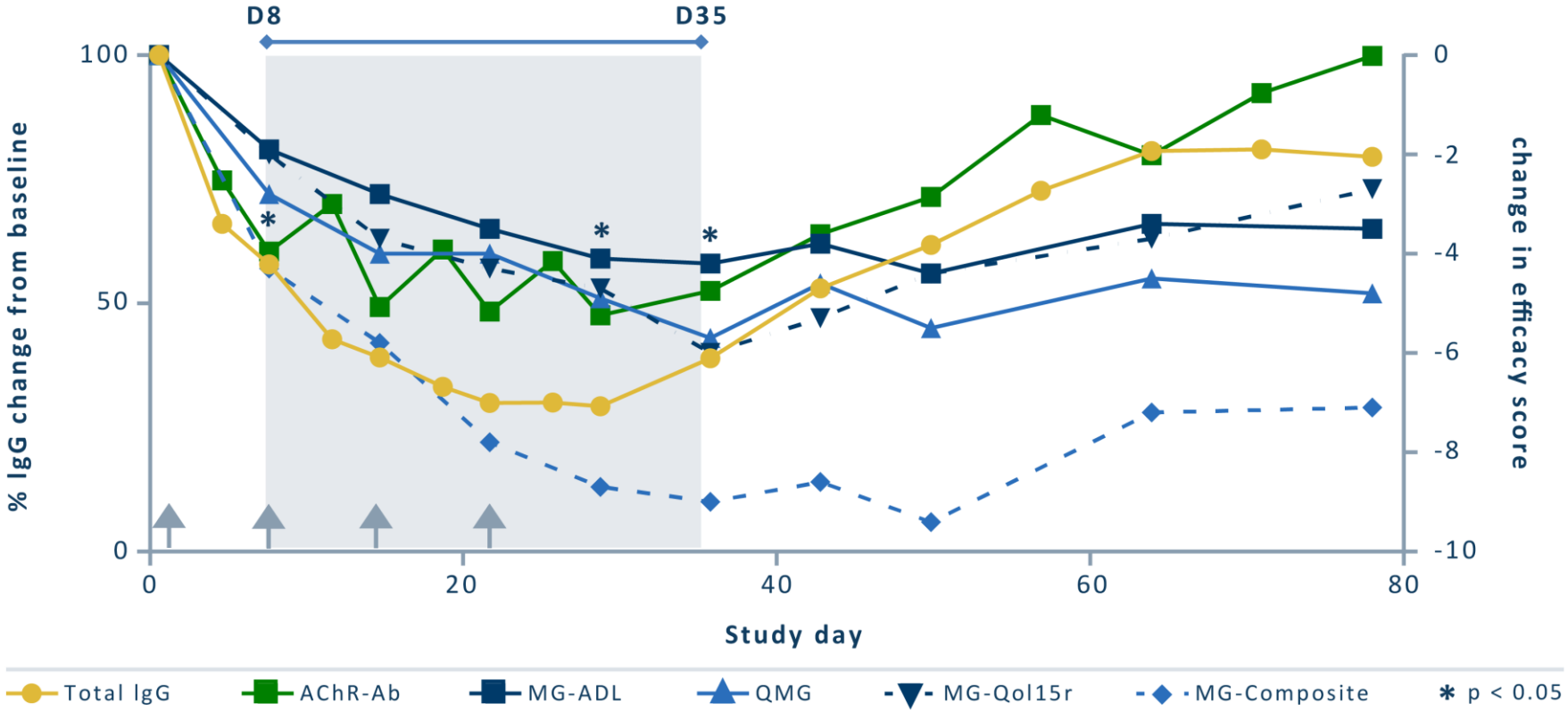
## Patients with MG-ADL ≥ 2 for a period of at least 6 weeks




- 83% of efgartigimod patients achieved clinically meaningful response (MG-ADL ≥2)
- 75% of efgartigimod patients had clinically meaningful and statistically significant improvement in MG-ADL score for at least 6 consecutive weeks versus 25% of patients on placebo

# Total and Pathogenic IgG Reduction Correlates with Clinical Improvements

Assessment for all efficacy scales in Phase 2




- Clinical improvement persists despite return of IgG levels
- Potential differentiation from PLEX where clinical benefit was reported to be lost 2-4 weeks after end of treatment

# Efgartigimod in Immune Thrombocytopenia – Phase 3 ADVANCE Trial to Start

## First of two registration Phase 3 trials to start in 2H19

- Randomized, double-blind, placebo-controlled, multicenter trial enrolling up to 158 adult patients with primary ITP
- Enrolling patients with platelet levels <30x$10^9$/L and stable dose and dosing frequency of SoC prior randomization
- 10 mg/kg IV dose over a 24-week treatment period
- Patients eligible to roll over into 1-year open-label extension trial

advance
immune thrombocytopenia study

**Primary objective**

**Efficacy (sustained platelet count of at least 50×$10^9$/L)**

**Secondary objectives**

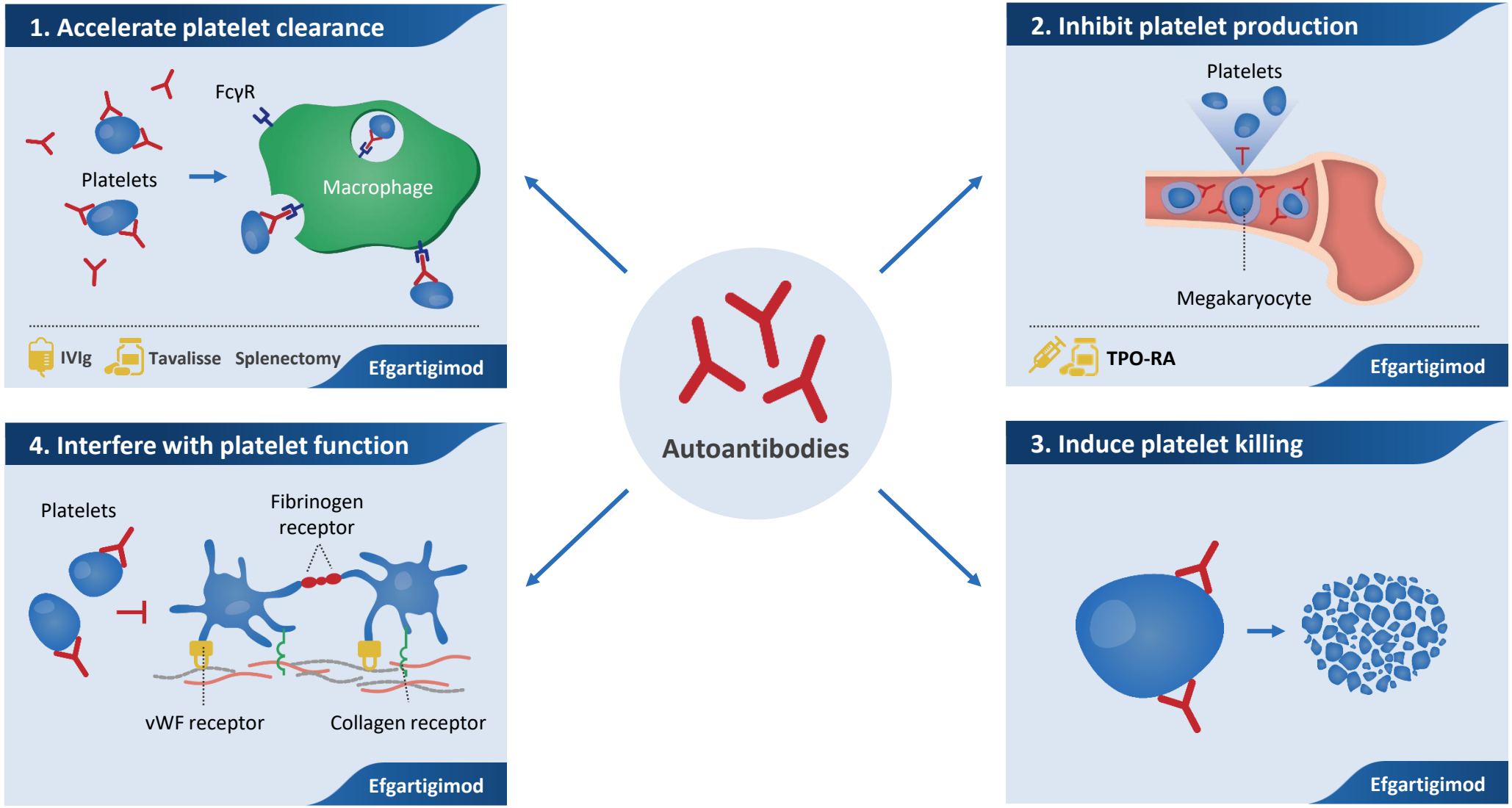
**Efficacy, safety, tolerability, incidence and severity of bleeding events and QoL**

Data from completed Phase 2 trial presented at the annual ASH conference demonstrating that:

- Treatment with efgartigimod resulted in clinically meaningful improvements in platelet counts and efgartigimod treatment showed a clear correlation between IgG reduction, platelet count improvement and bleeding event reduction
- Efgartigimod has a clean tolerability profile in line with HV study and treatment-emergent adverse events were balanced between active and placebo arms

# Efgartigimod in Immune Thrombocytopenia

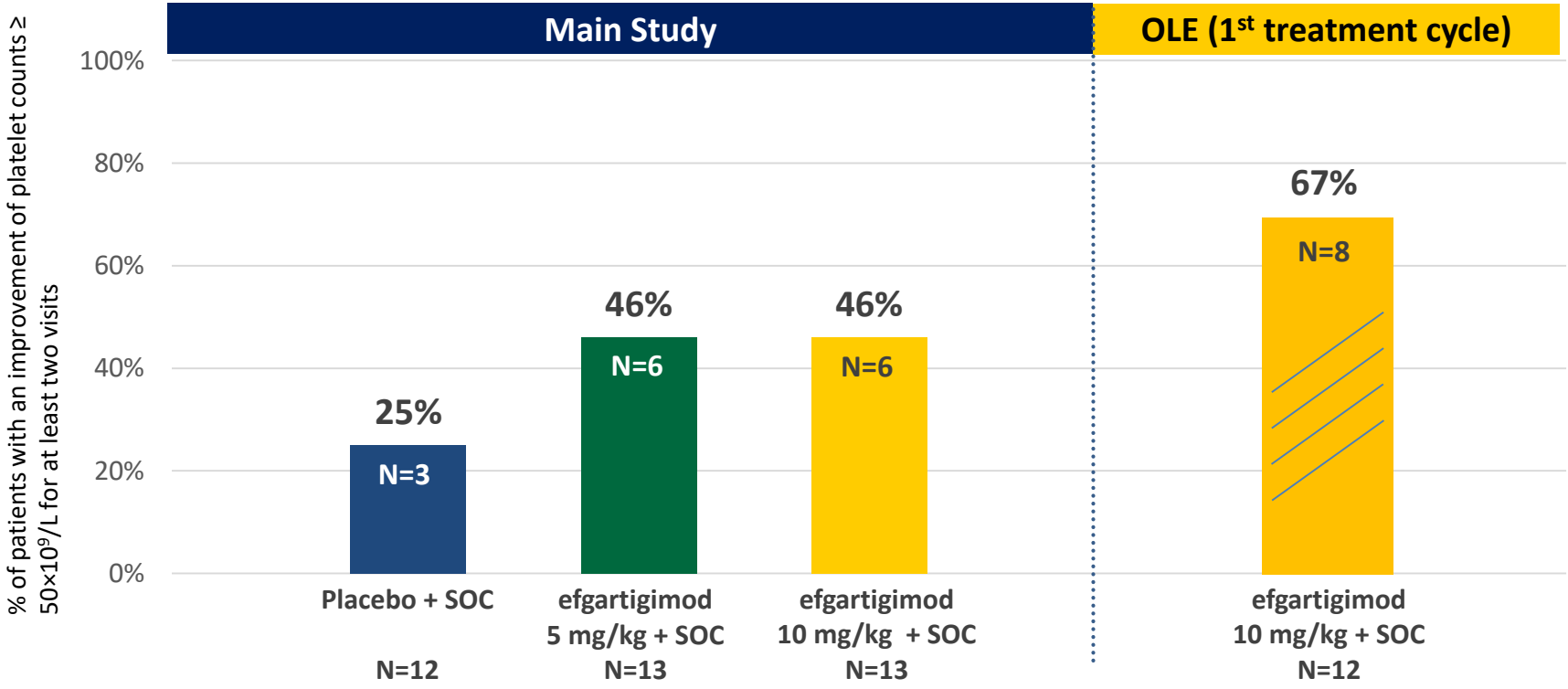
Targets all pathogenic autoantibody actions simultaneously and may limit therapeutic cycling



# Efgartigimod in Immune Thrombocytopenia – Strong Phase 2 Efficacy Results

## Strong improvement of platelet counts across doses

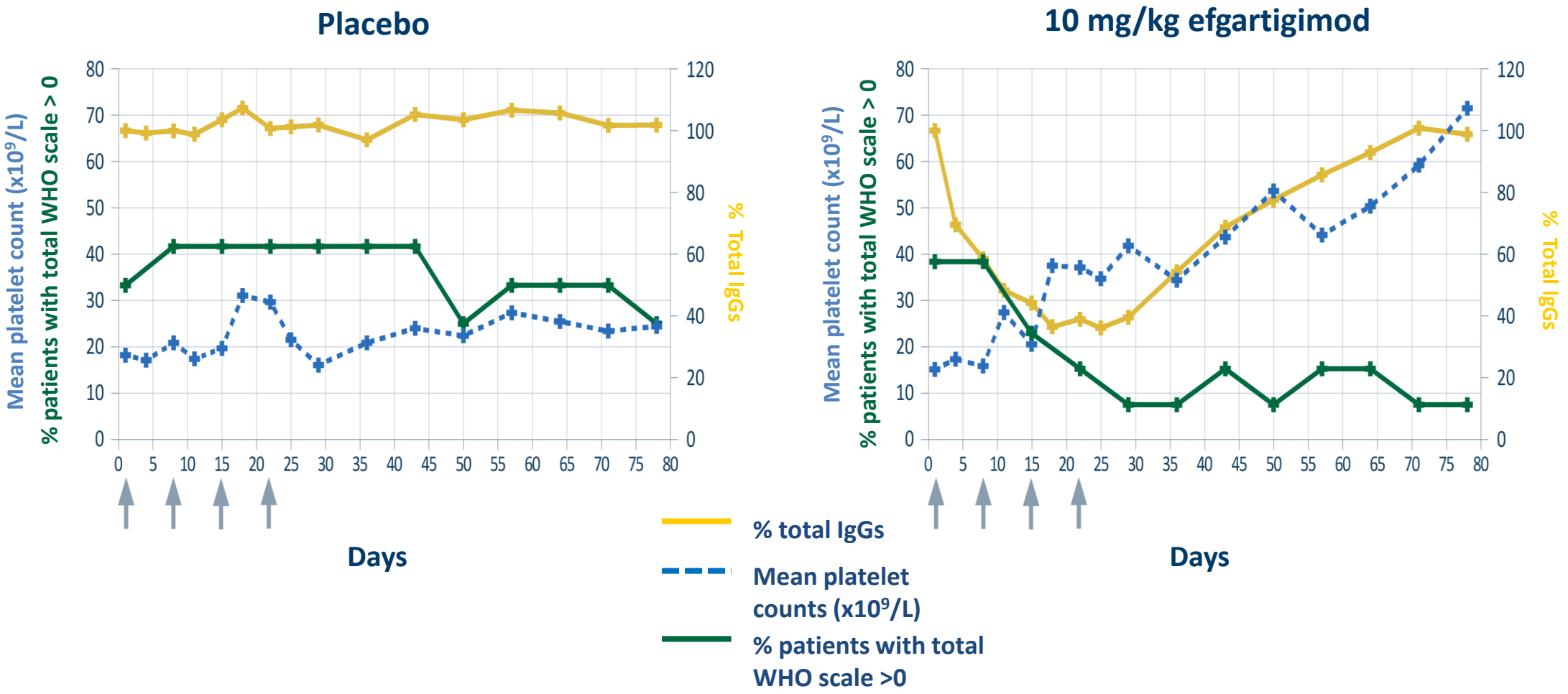
### 46-67% of patients achieving platelet counts of ≥ $50\times10^9$/L at least two times




- OLE acts as true fourth cohort since patients' platelets had to fall below $30x10^9$/L to be eligible for a treatment cycle; patients still in response from primary study were not eligible
- Responses seen across newly diagnosed (in 5mg/kg arm), persistent and chronic ITP patients

# Reduction of Total IgGs Correlates with Increased Platelet Counts and Reduced Bleeding Events

## Mean platelet counts versus total WHO scale versus total IgGs



# Efgartigimod in Pemphigus Vulgaris – Phase 2 Ongoing

## Cohort 3 enrolling – topline data expected 1H20

Phase 2, cohort 3 enrolling patients:

- Administration of extended dosing of efgartigimod
- To evaluate potential of efgartigimod to induce clinical remission

### Results from Cohort 1

**Rapid disease control in 4 out of 6 PV patients:**
- 3 within 1 week
- 1 within 4 weeks

**Patients with disease control:**
- Mean max reduction in Pemphigus Disease Area Index (PDAI) score: 55%
- Mean max decrease in pathogenic IgGs: 57%

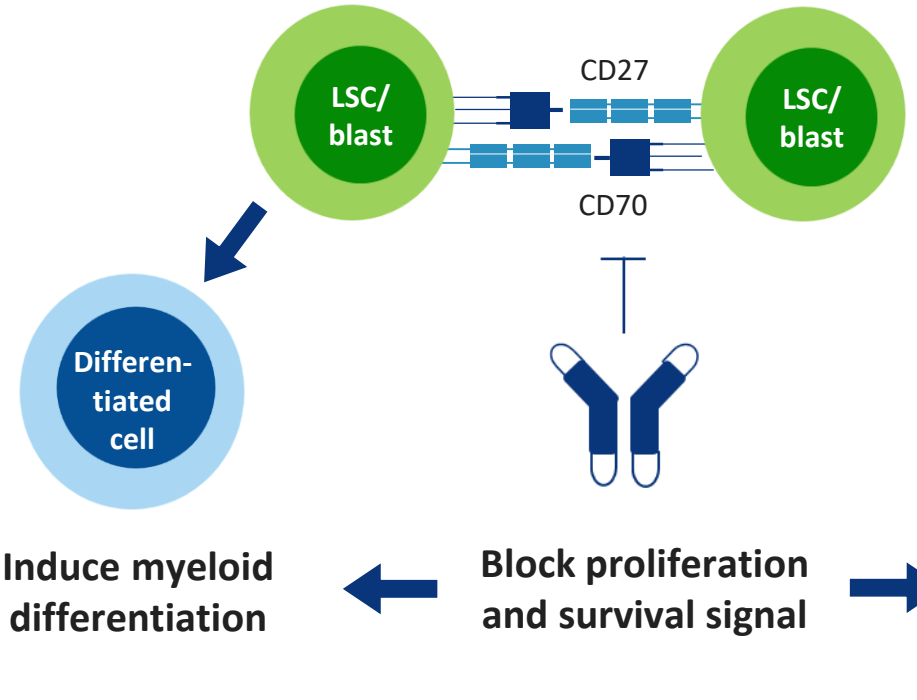
**Favorable tolerability profile**

**No meaningful anti-drug antibody signals (ADA) reported**

# Cusatuzumab – CD70 Inhibitor with First-In-Class Opportunity

Potential foundational, novel therapy for acute myeloid leukemia

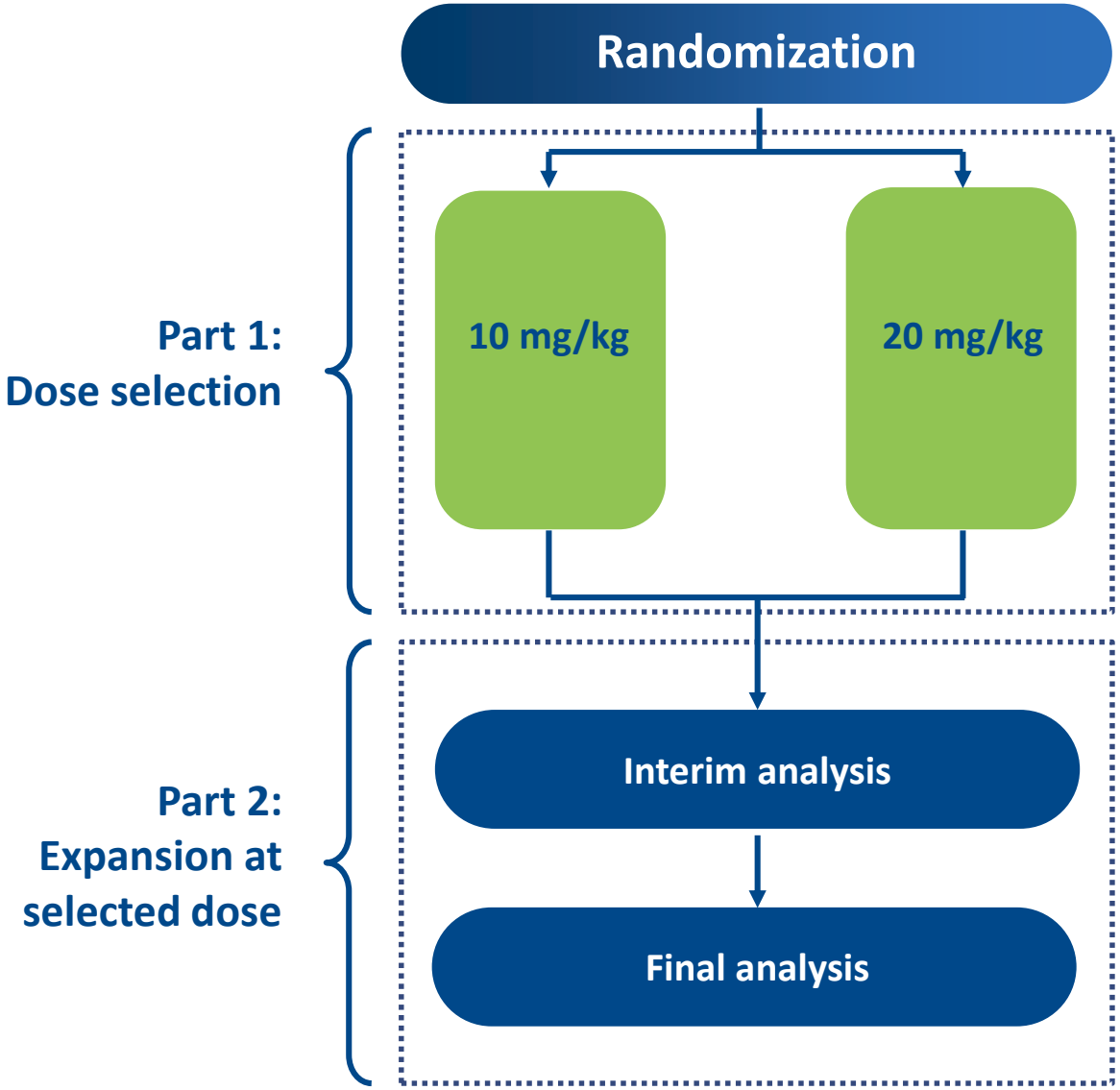
## Multiple MOA of Cusatuzumab




- Novel target and mechanism of action[1] (inhibition of CD70 pathway)
- Intrinsic activity shown as a single-agent in AML
- Potential for combination therapy[2]
- Phase 1/2 study: 92% ORR with 10/12 patients with CR/CRi after cusatuzumab treatment in combination with azacitidine (AZA) in newly diagnosed AML patients[3]
- IAP, Bern University – Prof. Ochsenbein

# Cusatuzumab in AML – Phase 2 to Start

Phase 2 and registration-directed trial in acute myeloid leukemia to start in 2H19




**Combination Therapy:** Cusatuzumab + Azacitidine

**Patient Population**: Newly diagnosed AML patients unfit for intensive chemotherapy (n= up to 150)

**Primary Objective**: To determine the efficacy (CR rate)

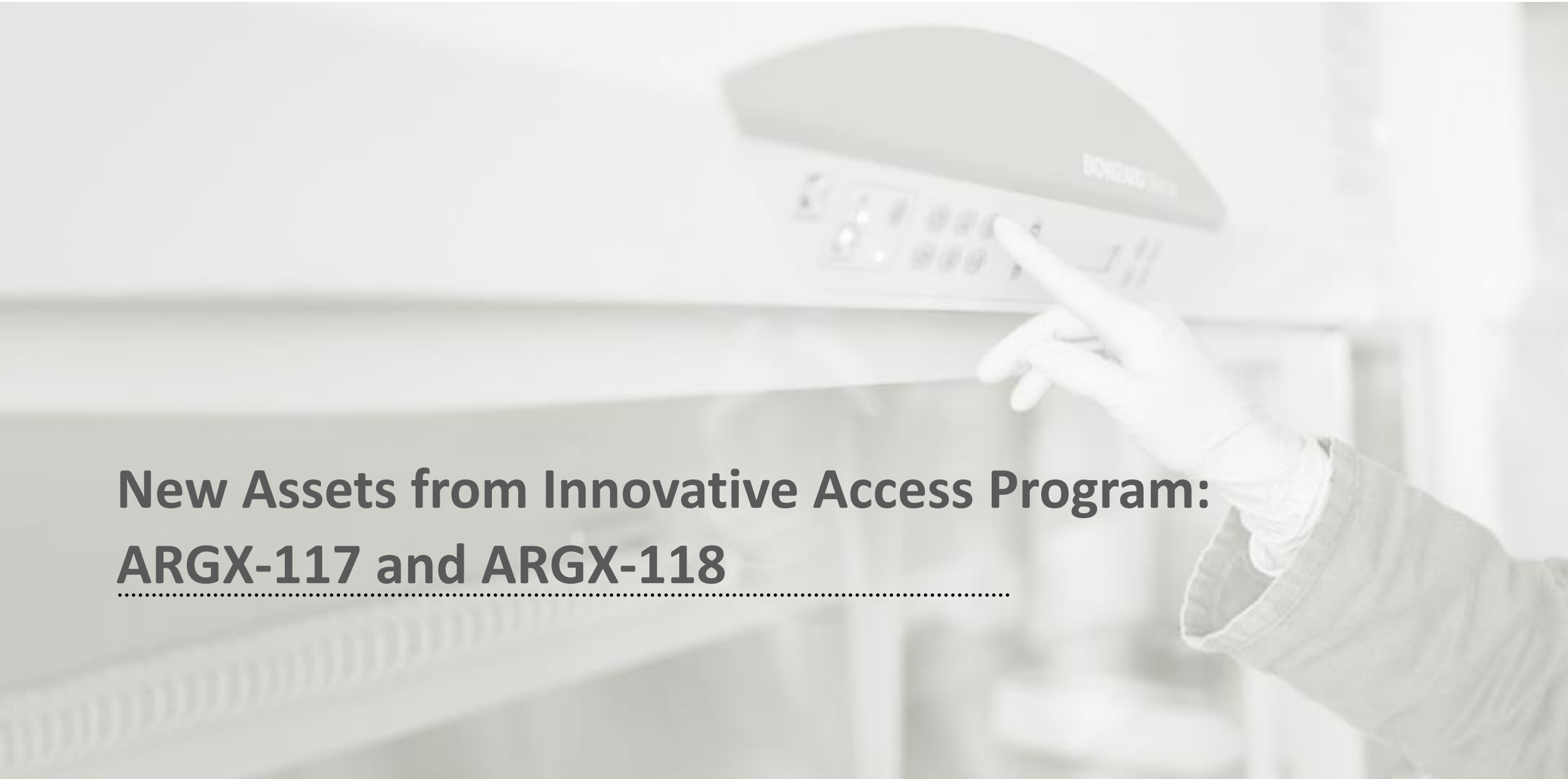
**Secondary Objectives**:

- ORR = (CR + CRh + CRi)
- Time to response and duration of response
- Event-free survival
- Overall survival
- Safety
- PK/immunogenicity
- MRD

**Anticipated** Phase 2 study start: second half 2019

# Cusatuzumab Strategic Alliance with Janssen Pharmaceuticals

| argenx objectives | Janssen alliance |
| --- | --- |
| Accelerate & broaden development plan | Joint development plan focused on AML, MDS and other heme malignancies |
| Secure strong financials | Upfront $300M + $200M equity @ 20% premium, $1.3B in milestones, double digit royalties OUS |
| Retain commercial upside | 50% of US economics on a royalty basis, up to 50% commercial efforts |



# New Assets from Innovative Access Program: ARGX-117 and ARGX-118

# Innovative Access Program

Pipeline recently expanded with addition of two preclinical assets

## Early Target Validation

**Power of SIMPLE Antibody™ technology**

→ Charcot-Leyden Crystal dissolving antibodies

**Unravelling novel airway inflammation biology**

→ Galectin-10 first novel airway inflammation target in decades

**ARGX-118**

## Jumpstart Product Development

**Power of NHance® technology and engineering know-how**

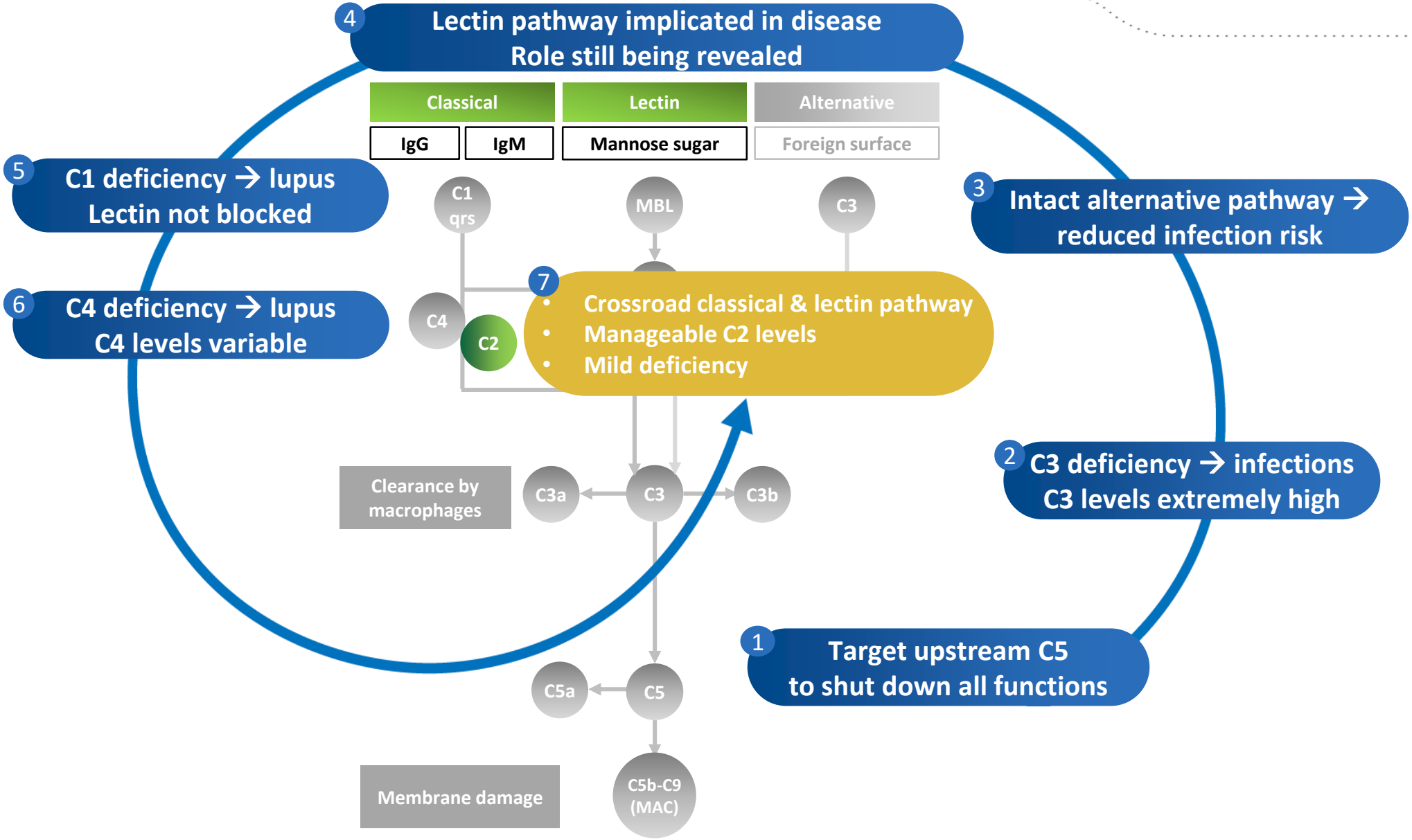
→ Turn unique mouse V-regions into highly differentiated product candidate

**Leveraging unique insights in complement disease biology**

→ Pipeline-in-product opportunity

**ARGX-117**

# Targeting C2 Preserves Key Complement Functionality



# ARGX-117: V and Fc Regions Act in Concert to Sweep C2

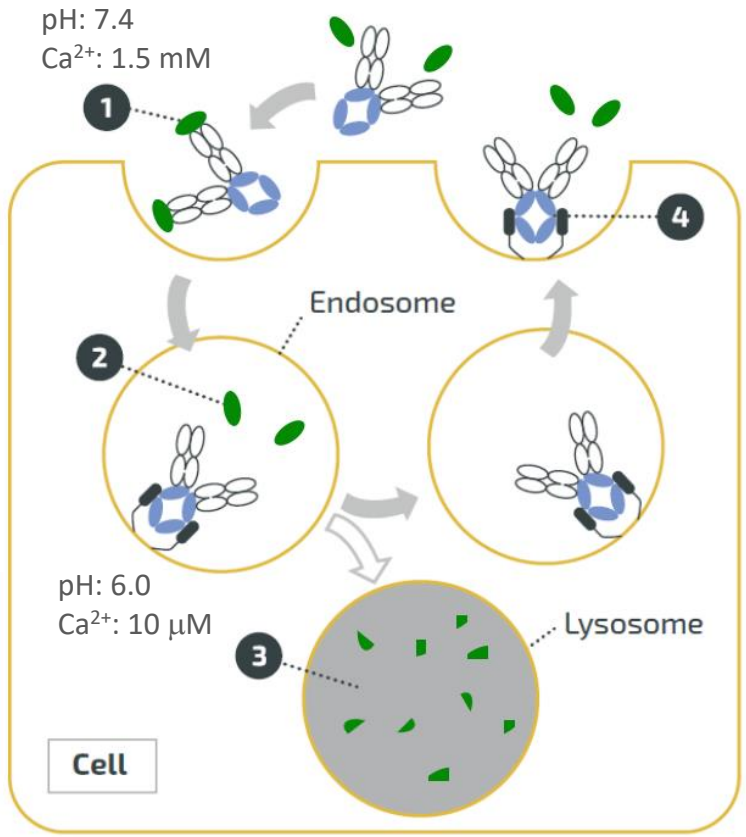
1 ARGX-117 binds C2 in circulation

2 ARGX-117/C2 complex taken up by pinocytosis entering endosome;

ARGX-117 releases C2 at lower pH in endosome

3 C2 is degraded in lysosome

4 ARGX-117 remains bound going back into circulation to bind to new C2 because of NHance® mutations



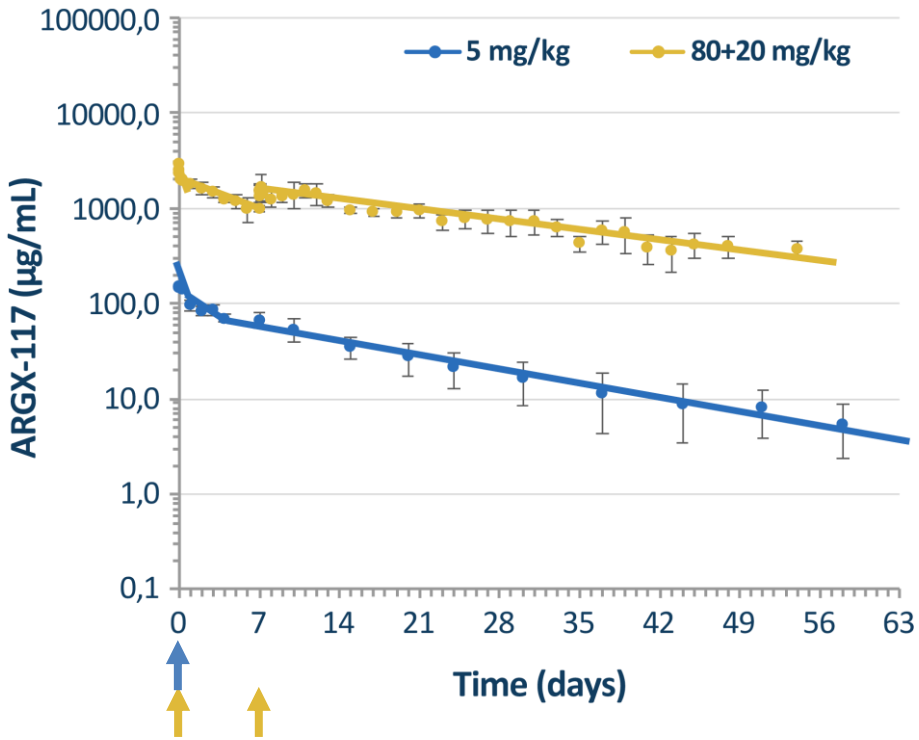

FcRn

ARGX-117

C2

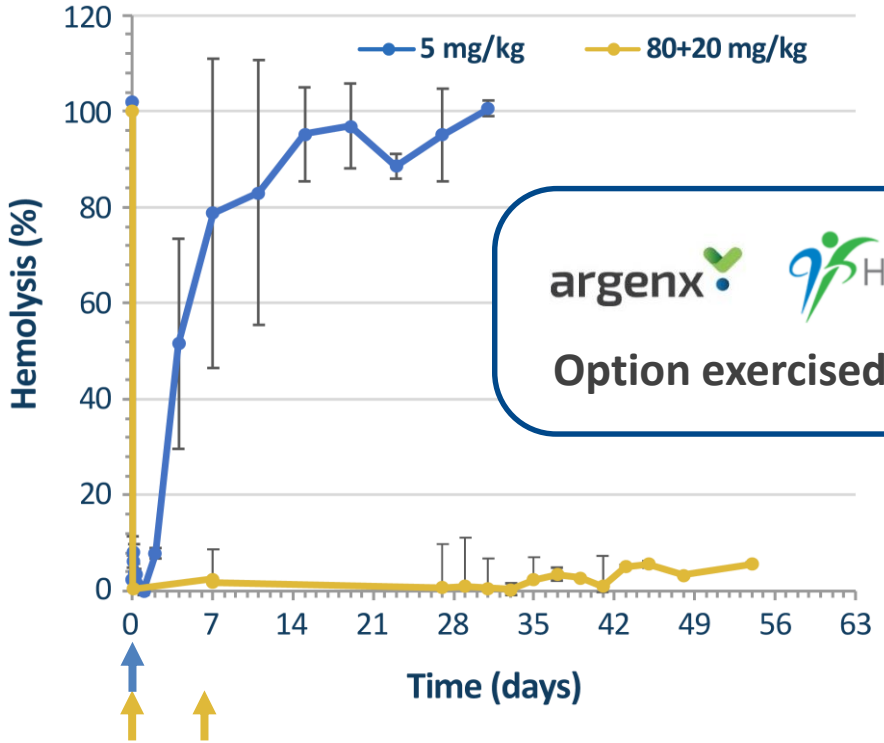
# ARGX-117: Dosing Optionality

## Pharmacokinetics




**Half-life ARGX-117: 2-3 weeks**

## Pharmacodynamics




argenx Halozyme

**Option exercised for C2**

**Blocking for 2 months after repeat dosing**

Cynomolgus monkey data

**C2 levels cynomolgus monkey = 4x human**

# Commercial

# Building Immunology Franchises

Neuro Inflammation | Poly Neuropathies | Myositis

**Expansion Opportunities**

MG | CIDP | MMN

**Neuro-muscular**

BMT | AIHA | ANCA | Leukemia Lymphomas

**Expansion Opportunities**

MALIGNANT HEME

AML | MDS

BENIGN HEME

ITP

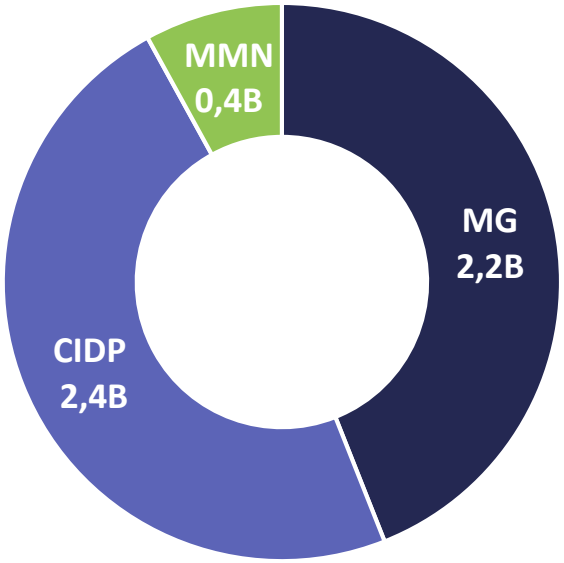
janssen

50/50

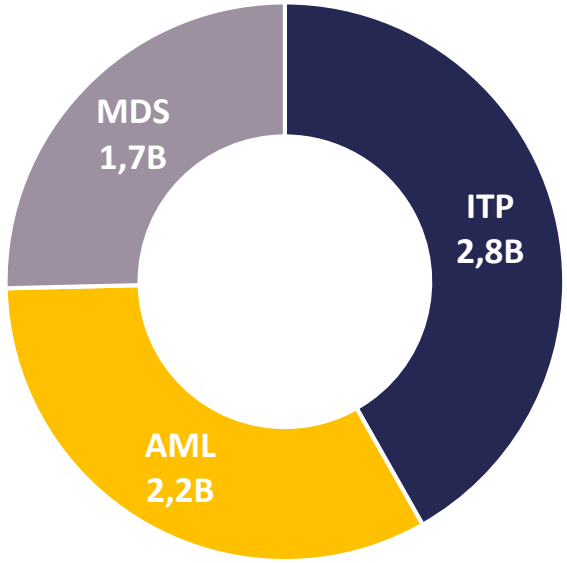
**Hem/Onc**

# Franchises Sit in High-Value Rapid-Growth Global Markets

**Neuromuscular**
**>$5B (CAGR ~10%+)**




**Hem/Onc**
**~$7B (CAGR ~10%)**




- ***~370,000 patients***
- Double digit CAGR

Efgartigimod ARGX-117 Cusatuzumab


# Wedbush Healthcare Conference
# August 2019