

argenx 

Welcome & Opening Remarks

Beth DelGiacco /// Vice President, Corporate Communications & Investor Relations

Forward Looking Statements

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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. 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. These forward-looking statements can be identified by the use of forward-looking terminology, including the terms "advance," "broaden," "build," "develop," "expand," "grow," "predict," "potential," "reach," "start," "seek," "vision," and "will," and include statements argenx makes regarding the potential of its new pipeline candidates, including ARGX-213 and ARGX-121; future phases of ongoing product candidate development; the anticipated timing of argenx's clinical trials, including the anticipated timing of the end of the Phase 2 ARDA clinical trial and the initiation of the Phase 3 clinical trial for empasiprubarb in MMN, the anticipated timing of the Phase 2 EMPACIFIC clinical trial, the anticipated timing of the analysis for the Phase 2 ALKIVIA clinical trial, the anticipated timing of the initiation of the Phase 3 clinical trial for efgartigimod in SjD, the anticipated timing of the initiation of the Phase 1b and Phase 2a clinical trials for ARGX-119 in CMS and ALS, respectively, the anticipated timing of the initiation of the Phase 1 clinical trial for ARGX-213, and the anticipated timing of the initiation of the Phase 1 clinical trial for ARGX-121; the timing and outcome of regulatory filings and regulatory approvals, including the anticipated timing of the clinical trial applications for ARGX-121 and ARGX-213; the number of patients that its products will reach in 2030; the size and growth of the market for its products, including the growing MG opportunity, the in-market opportunity when evaluating ocular and seronegative MG, and the MMN, SjD and TED opportunities; its future position as a market leader among branded biologics; its thought leadership in the scientific community; its trajectory to be a leading autoimmune franchise; the outcome and findings of its various studies, including the findings of the iMMersioN clinical trial; its goals and visions for its future advancement, including its vision for 2025 and 2030; its capabilities to scale; and the number of its products and the number of indications those products will have. By their nature, forward-looking statements involve risks and uncertainties and readers are cautioned that any such forward-looking statements are not guarantees of future performance. argenx's actual results may differ materially from those predicted by the forward-looking statements as a result of various important factors, including the results of argenx's clinical trials; expectations regarding the inherent uncertainties associated with the development of novel drug therapies; preclinical and clinical trial and product development activities and regulatory approval requirements in products and product candidates; the acceptance of argenx's products and product candidates by patients as safe, effective and cost-effective; the impact of governmental laws and regulations on our business; disruptions caused on our reliance of third parties suppliers, service providers and manufacturing; inflation and deflation and the corresponding fluctuations in interest rates; and regional instability and conflicts. A further list and description of these risks, uncertainties and other risks can be found in argenx's U.S. Securities and Exchange Commission (the "SEC") filings and reports, 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. Given these uncertainties, the reader is advised not to place any undue reliance on such forward-looking statements. These forward-looking statements speak only as of the date of publication of this document. argenx undertakes no obligation to publicly update or revise the information in this presentation, including any forward-looking statements, except as may be required by law.

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Agenda

Welcome and Opening Remarks	Beth DelGiacco
argenx Vision 2030	Tim Van Hauwermeiren
Immunology Innovation	Peter Ulrichts, Karen Silence
Clinical Development	Luc Truyen
Myositis	Leentje De Ceuninck
Sjögren's Disease	Julie Jacobs
Sjögren's Disease KOL Panel	Julie Jacobs, Prof. Simon Bowman (Moderated by Luc Truyen)
Q&A Session 1	argenx Management Team
BREAK	
Phase 2 ARDA Study (MMN)	Inge Van de Walle, Jeff Guptill
MMN KOL Panel	Dr. Patrick Kwon, Jeff Guptill (Moderated by Luc Truyen)
Sustainable Commercial Engine	Karen Massey
Q&A Session 2	argenx Management Team

argenx Leadership Here Today



Tim Van Hauwermeiren
Chief Executive Officer



Karen Silence Ph.D.
Head Preclinical Product
Development



Beth DeGiaccio
Vice President, Corporate Communications
Investor Relations



Luc Truyen M.D., Ph.D.
Chief Medical Officer



Peter Ulrichs Ph.D.
Chief Scientific Officer



Karen Massey
Chief Operating Officer



Leentje DeCeuninck Ph.D.
Senior Clinical Scientist



Jeff Guptill, M.D.
Neuromuscular Franchise Lead,
Clinical Development



Julie Jacobs Ph.D.
Principal Scientist



Inge Van de Walle Ph.D.
Research Fellow

Thought Leaders Here Today



Simon Bowman, Ph.D., M.B.B.S., F.R.C.P.

Institute of Inflammation and Ageing,
University of Birmingham



Patrick Kwon, M.D.

Clinical Associate Professor, Neurology,
New York University Grossman School of Medicine



Key Themes For Today

Our innovation model

Leadership in FcRn

Expansion of our immunology pipeline

Setting a new standard in MG and CIDP

Next wave of efgartigimod indications

Building weight behind empasiprubart

Vision 2030 - path to 50,000 patients

Vision 2030

Tim Van Hauwermeiren /// Chief Executive Officer

Vision 2030

5

New Molecules
in Phase 3

10

Labeled
Indications

50k

Patients on
Treatment

COMMITMENT TO OUR TRANSFORMATION MISSION

Continuous Pipeline of Innovation

Leadership in FcRn

Disciplined Scaling

argenx 

Est. 2008
argenx



Entrepreneurial spirit – calculated risk based on data

Immunology innovation through model of **co-creation**

Execution excellence

Our Understanding of Human Immunology is Growing Exponentially



Our Innovation Playbook

**Novel Disease
Biology Insights**

**Foundational
Immune
Targets**

**Best-in-Field
Antibody
Engineering**

**First-in-Class
Antibodies**

**Pipeline-in-
a-Product
Development**

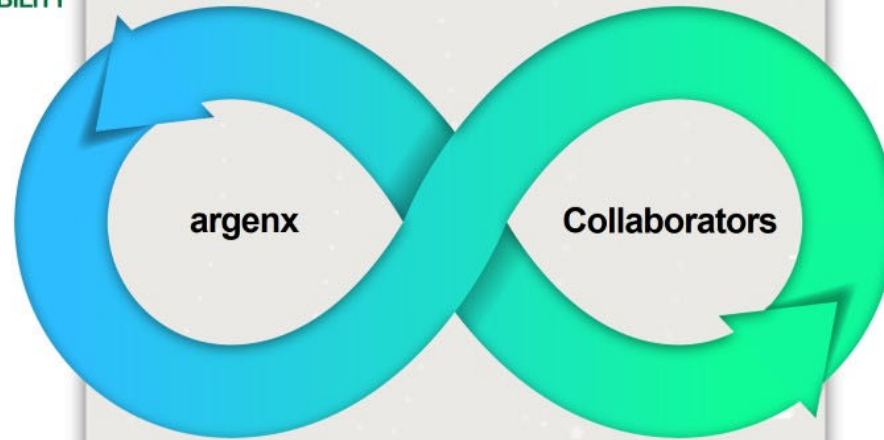
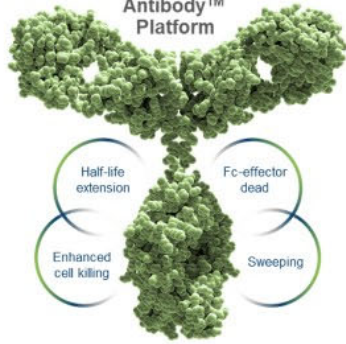
**Differentiated
Patient
Outcomes**

Co-Creation is Our Innovation Formula

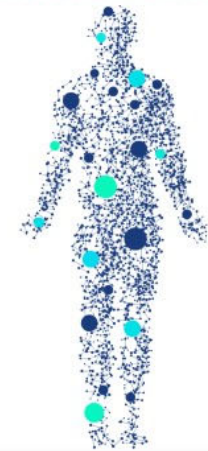
WORLD CLASS ANTIBODY ENGINEERING CAPABILITY

V-REGION CAPABILITIES

SIMPLE
Antibody™
Platform



DISEASE BIOLOGY INSIGHTS



Our Innovation Model Has A Strong Track Record

IL6

CD70

CMET

IL22R

FcRn

GARP

ApoC3

C2

Galectin-10

MuSK

IgA

'ARGX-220'

8/12 demonstrated human POC

9 first-in-class targets

5 partnered

Broad applicability **across 35+ indications**

Innovation Through Co-Creation Exists Across argenx

DISCOVERY

Logos of academic and research institutions in the Discovery phase include: iip (Innovation Integration Program), LU (Leiden University), M C (Maastricht University), NYU (New York University), UMC Utrecht, Inserm, Aarhus University, UHASSELT, de Duve Institute, Amsterdam UMC, Massachusetts General Hospital, UT Southwestern Medical Center, and Sanquin.

DEVELOPMENT

Logos of industry and research partners in the Development phase include: elektrofi, IQVIA, zaiLab., adapt myasthenia gravis study, arda (Acute Relapsing Autoimmune Neuropathy Study), Halozyme, adapt myasthenia gravis study, adhere (Chronic Inflammation in Myasthenia Gravis Study), and alkivia (Small Molecule Therapy Study).

COMMERCIAL

Logos of commercial partners and products in the Commercial phase include: MG United by argenx, SHINING THROUGH CIDP, VYVGART Hytrulo (efgartigimod alfa and hyaluronidase-qvfc) Subcutaneous Injection, 100 mg/mL and 2000 mg/mL vial, and My VYVGART Patch.

Successful Execution Of Our Vision 2025

Growing autoimmune market

Efgartigimod available globally

Vibrant franchises

Efgartigimod in development in 15 indications

ARGX-117 in late-stage trials

Proof-of-concept in ARGX-119

New asset each year from IIP

Committed to our Patients and their Communities

Rooted in Science through our IIP

Enviably Immunity Pipeline

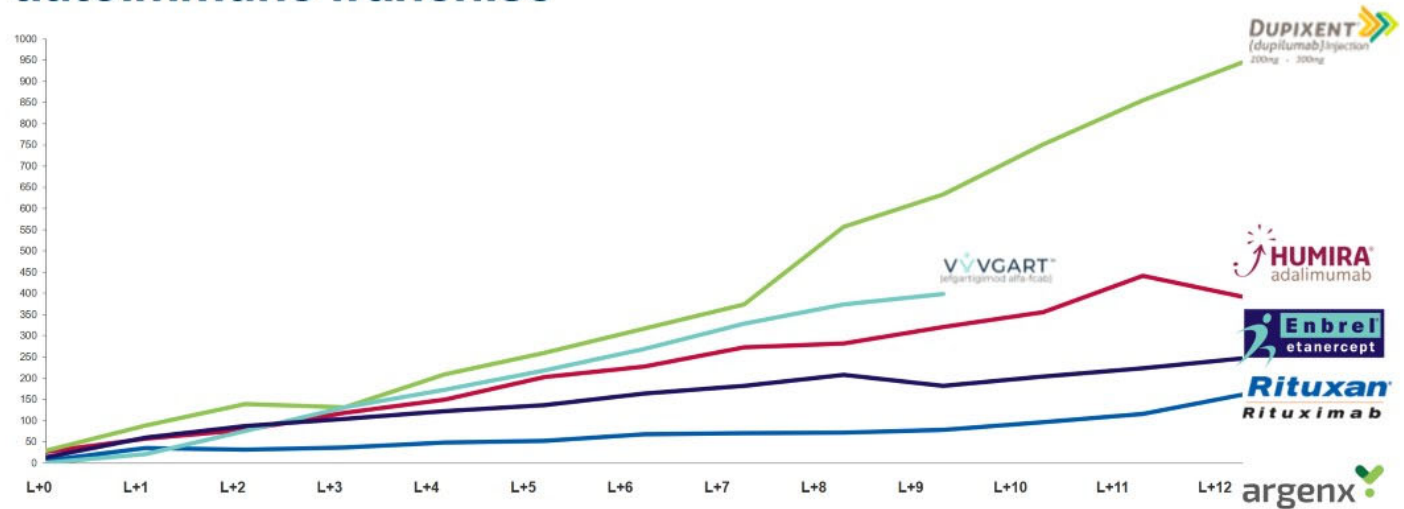
VYVGART is a Global Blockbuster

VYVGART generated >\$1B
in second year of launch

Approved in 3
indications globally

Leading market share among
MG branded biologics

On launch trajectory to leading autoimmune franchise



Reaching Patients Globally with VYVGART Franchise

>10,000 patients on treatment¹

VYVGART and VYVGART Hytrulo² approved across 3 continents within one calendar year

Reaching Patients Across the Globe



1. Patients on treatment globally as of 1Q 2024
2. VYVGART Hytrulo is marketed as VYVGART-SC in Europe and VYVDURA[®] in Japan

Staying True to our Scientific Roots

From IIP to marketplace, science
is our common language

Robust patent portfolio

Advanced our scientific expertise
with peer reviewed publications
in top medical journals



nature
International weekly journal of science



THE LANCET
Neurology



Neurology®

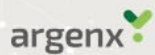


 **frontiers**
in Immunology

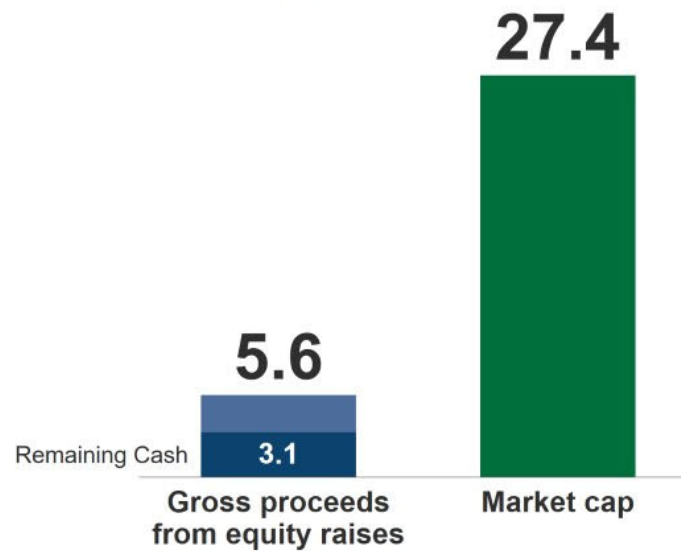
Creating Superior Shareholder Value on our Path to Self-Sustainability

Rapid transition to sustainable company

Disciplined scaling



Total Shareholder Return since IPO in 2014
\$B



argenx TSR
~5,000%

Vision 2030

5

New Molecules
in Phase 3

10

Labeled
Indications

50k

Patients on
Treatment

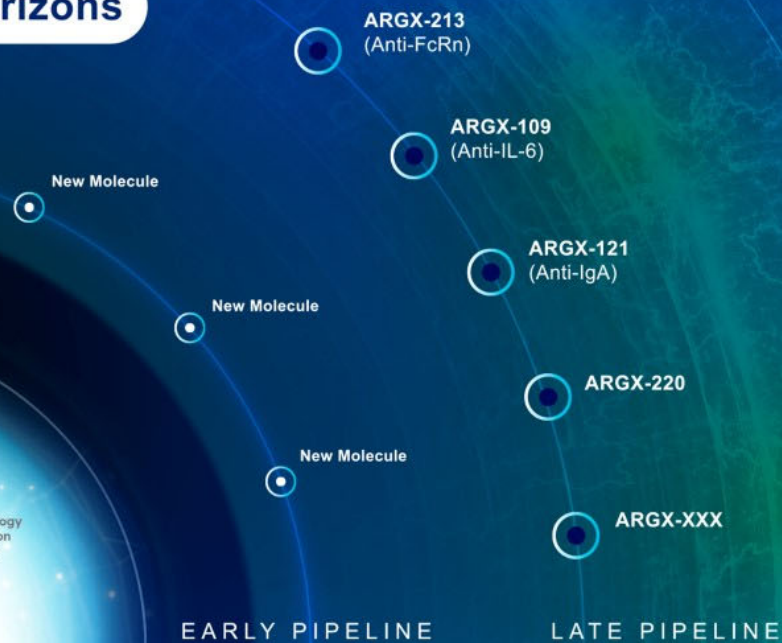
COMMITMENT TO OUR TRANSFORMATION MISSION

Continuous Pipeline of Innovation

Leadership in FcRn

Disciplined Scaling

Our Horizons



5 Additional
Molecules
in Phase 3

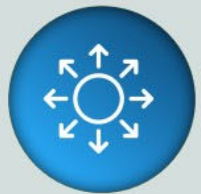
10 Labeled Indications

In-Market Expansion



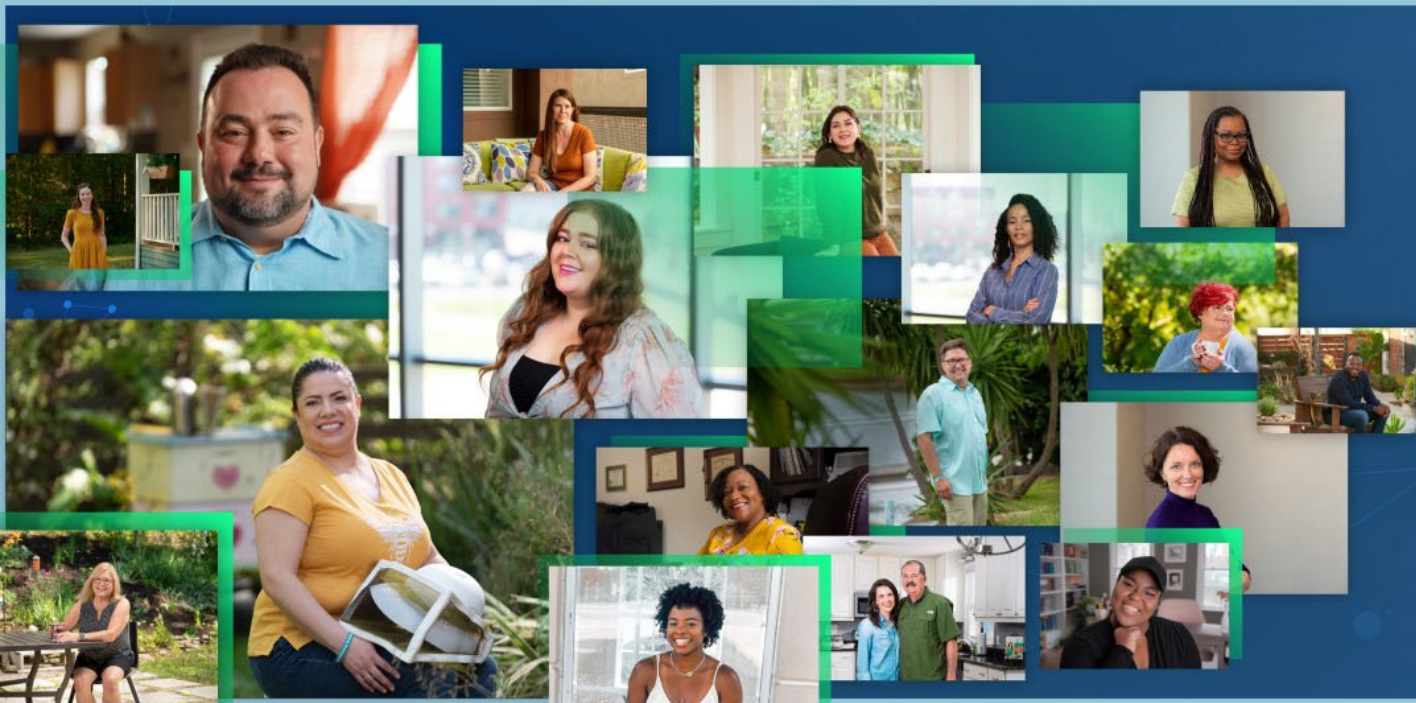
- gMG ✓
- Sn MG
- ITP ✓
- Ocular MG
- CIDP ✓

Next Wave of Potential Launches



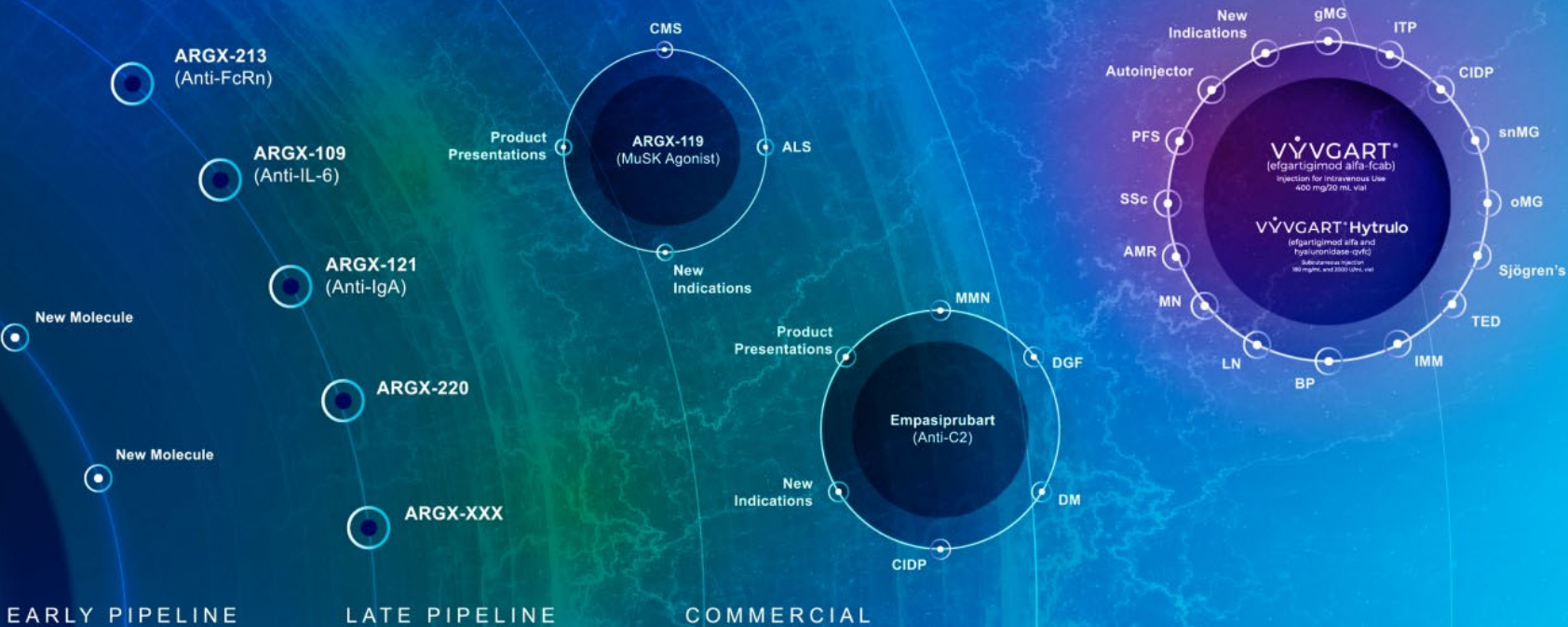
- TED
- DM (empa)
- CMS (119)
- SjD
- MMN (empa)
- BP
- Myositis [3]
- CIDP (empa)

✓ Currently approved indications ● VYVGART ● Empasiprubar ● ARGX-119



Innovation has no meaning unless it reaches patients and provides real benefit

Our Horizons



argenx 

A network diagram background with blue and white nodes connected by lines, set against a dark blue gradient. The nodes are scattered across the slide, with a denser cluster on the right side.

Blueprint for Innovation Next Wave of First-in-Class Immunology Targets

Peter Ulrichs /// Chief Scientific Officer

Immunology Innovation Program: Model of Co-Creation

ARGX-113

Efgartigimod

Foundational
Immune Targets

ARGX-117

Empasiprubart

Best-in-Class
Engineering
First-in-Class
Antibodies

ARGX-119

Pipeline
Development
Differentiated Patient
Outcomes

ARGX-117

Unraveling Central Role of C2 in Complement Cascade

Novel Disease
Biology Insights

Best-in-Field
Antibody
Engineering

Pipeline-in-
a-Product
Development

Novel Disease
Biology Insights

Unique Positioning
of C2

MMN Disease
Biology

Foundational
Immune Targets

C2

Best-in-Field
Antibody
Engineering

Potent C2 Sweeping

Long Half-Life

First-in-Class
Antibodies

Empasiprubart

Pipeline-in-
a-Product
Development

MMN, DGF, DM, CIDP

Differentiated Patient
Outcomes

POC ARDA Data

Unique complement toolkit

C2-KO and human C2-transgenic mice

>30 complement assays in house across different species

Various species cross-reactive anti-C2 mAbs for translational models

EMPASIPRUBART

**INNOVATION
ECOSYSTEM**

Extensive network of experts



University of Glasgow

Cedars Sinai



UMC Utrecht

AARHUS UNIVERSITY



KU LEUVEN

L U M C Leids Universitair Medisch Centrum

UNIVERSITY OF LEICESTER

VIB

Advancing science

JACI The Journal of Allergy and Clinical Immunology

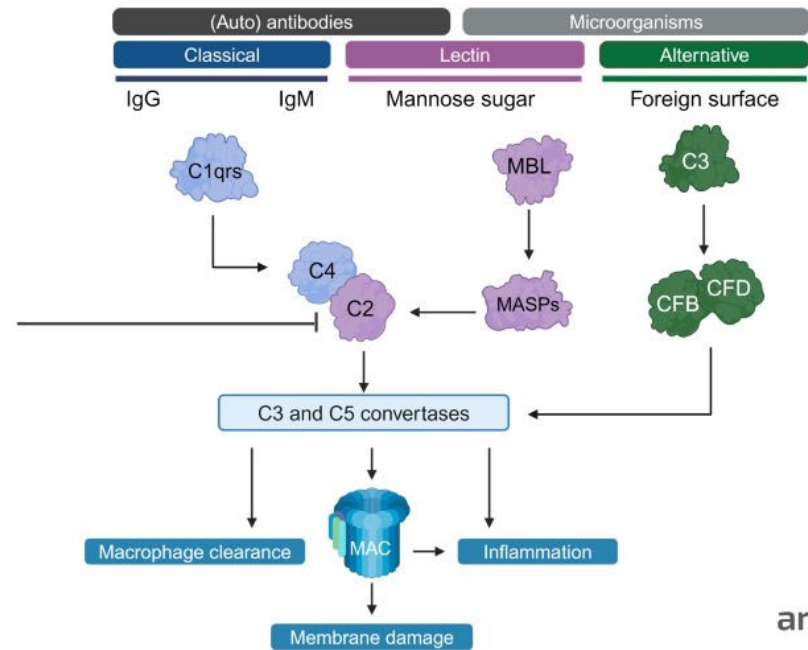
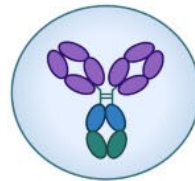
BRAIN COMMUNICATIONS

AMERICAN ACADEMY OF NEUROLOGY

Empasiprubart in Action

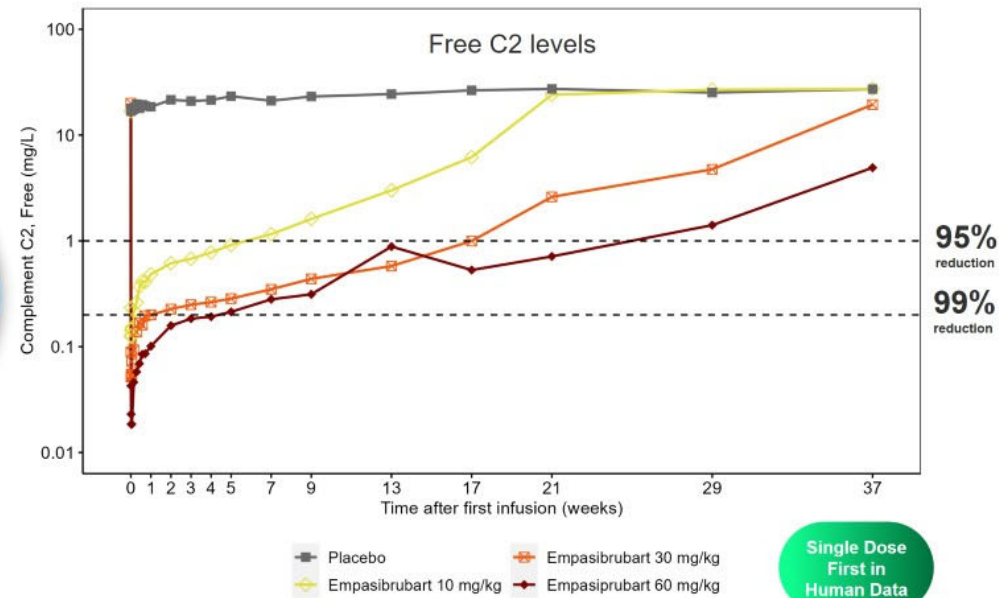
C2 is Uniquely Positioned in Complement Cascade

Empasiprubart



Empasiprubart Demonstrates Long Half-life and Sustained Pharmacodynamic effect

Sustained reduction in free C2 levels by 95% for > 100 days as of 30 mg/kg dose



Immunology Innovation Program: Model of Co-Creation

**Novel Disease
Biology Insights**

Foundational
Immune Targets

**Best-in-Field
Antibody
Engineering**

First-in-Class
Antibodies

**Pipeline-in-
a-Product
Development**

Differentiated Patient
Outcomes

Immunology Innovation Program: Model of Co-Creation

ARGX-113

Efgartigimod

Foundational
Immune Targets

ARGX-117

Empasiprubart

Best-in-Class
Engineering
First-in-Class
Antibodies

ARGX-119

Pipeline
Development
Differentiated Patient
Outcomes

ARGX-119

Strengthening the Neuromuscular Junction through MuSK Activation

Novel Disease
Biology Insights

Best-in-Field
Antibody
Engineering

Pipeline-in-
a-Product
Development

Novel Disease
Biology Insights

Pioneering MuSK Biology

Foundational
Immune Targets

MuSK

Best-in-Field
Antibody
Engineering

Best-in-Field Antibody Engineering

First-in-Class
Antibodies

ARGX-119

Pipeline-in-
a-Product
Development

CMS, ALS
Studies

Innovative Endpoint
with MScan

Differentiated Patient
Outcomes

**Phase 1 Data Support
POC Studies**

Unique MuSK toolkit

MuSK phosphorylation assays

AChR clustering assays

Various binding assays

Various in vivo neuromuscular disease models including DOK7 CMS mice

Human in vitro ALS NMJ co-cultures

ARGX-119

INNOVATION ECOSYSTEM

Extensive network of experts

LU
MC Leids Universitair
Medisch Centrum

NYU Langone
Health

UNIVERSITY OF
OXFORD

TRICALS
The highway towards a cure

Université
de Montréal

neuro
McGill Neurological
Institute Hospital

CHEO

MGH MASSACHUSETTS
GENERAL HOSPITAL

RAYA
THERAPEUTIC

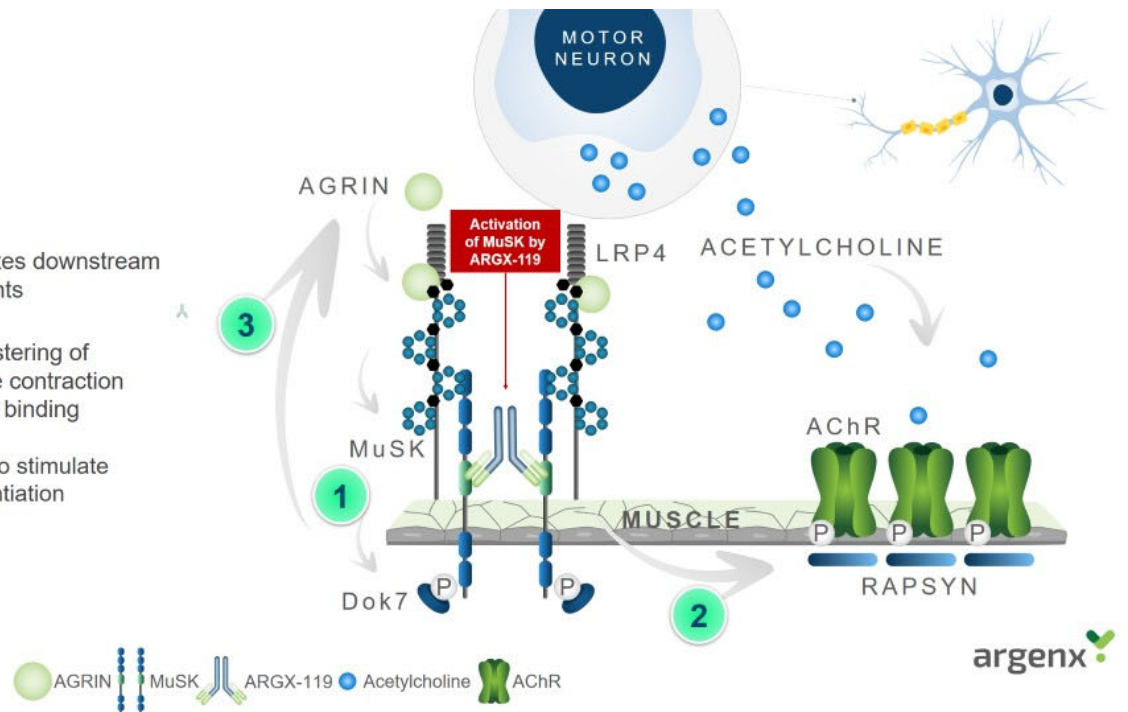
Advancing science

nature

Science
Translational
Medicine

ARGX-119 Boosts Functioning of NMJs by Improving AChR Clustering

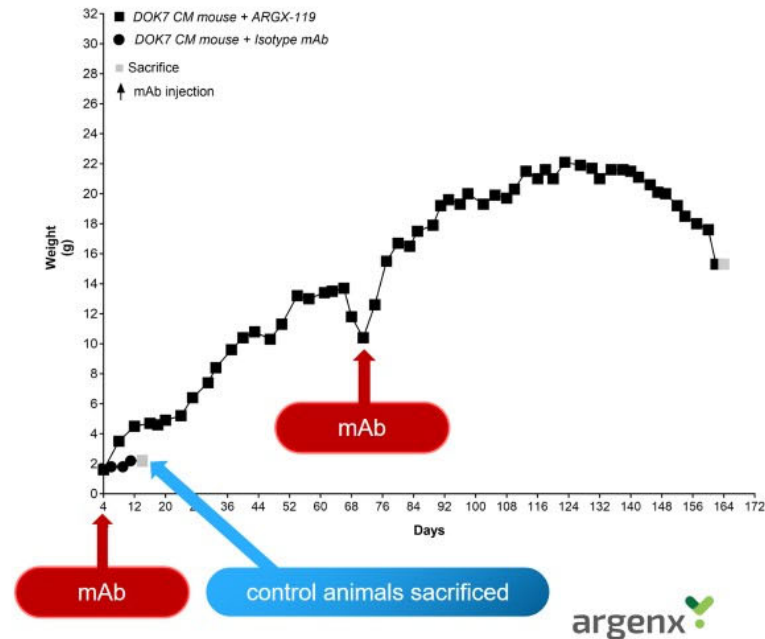
- 1 MuSK phosphorylates downstream signaling components
- 2 MuSK-induced clustering of AChRs and muscle contraction upon Acetylcholine binding
- 3 Retrograde/signal to stimulate presynaptic differentiation



CMS Rationale

Early Neonatal Lethality and Disease Relapse are Rescued by ARGX-119 in DOK7 CMS mice

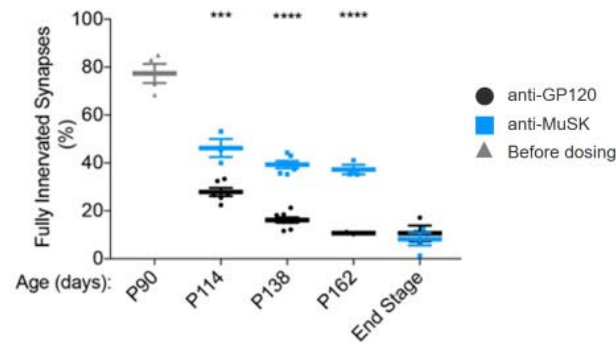
- 1 Diminished MuSK phosphorylation in DOK7 CMS
- 2 Leads to lethal weakness of diaphragm muscles
- 3 MuSK activation by ARGX-119 rescues phenotype



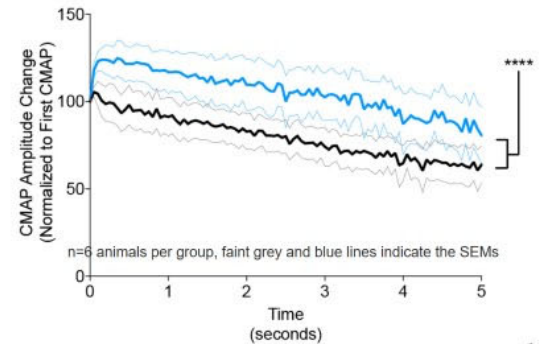
ALS Rationale

Activation of MuSK Signaling Slows Muscle Denervation and Improves Motor Function

Slowdown of muscle denervation



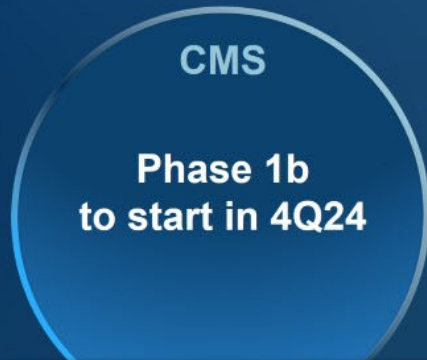
Improving motor system output



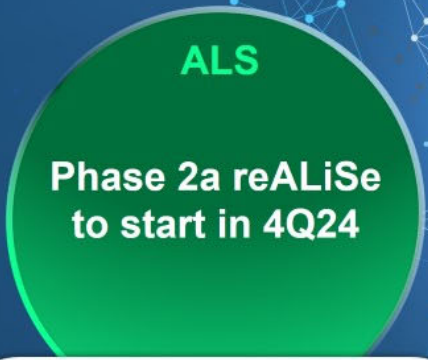
In vivo model show: Delayed disease onset | Improvement in survival

Reference: Cantor et al. 2018; Pérez-García et al. 2012; argenx internal data;

Path Forward for ARGX-119



Proof of Biology
Intra-Patient Dosing



Innovation Within Discovery
MScan
(MScan-derived Motor Unit Number)



Immunology Innovation Program: Model of Co-Creation

**Novel Disease
Biology Insights**

Foundational
Immune Targets

**Best-in-Field
Antibody
Engineering**

First-in-Class
Antibodies

**Pipeline-in-
a-Product
Development**

Differentiated Patient
Outcomes

Immunology Innovation Program: Model of Co-Creation

ARGX-113

Efgartigimod

Foundational
Immune Targets

ARGX-117

Empasiprubart

Best-in-Class
Engineering
First-in-Class
Antibodies

ARGX-119

Pipeline
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ARGX-113

Leadership in FcRn

**Novel Disease
Biology Insights**

**Best-in-Field
Antibody
Engineering**

**Pipeline-in-
a-Product
Development**

* as of Q1 2024

Novel Disease
Biology Insights

Pioneering FcRn Biology

Foundational
Immune Targets

FcRn

Best-in-Field
Antibody
Engineering

Unique Modulation of FcRn

First-in-Class
Antibodies

Efgartigimod

Pipeline-in-
a-Product
Development

Pipeline-in-a-Product Development

Differentiated Patient
Outcomes

**>10,000* patients
on VYVGART**

* as of Q1 2024

Unique FcRn toolkit

Differentiating binding assays

Predictive cellular assays

Expert structural biology

Innovative hFcRn/hAlbumin
transgenic mouse models

EFGARTIGIMOD

INNOVATION ECOSYSTEM

Extensive network of experts

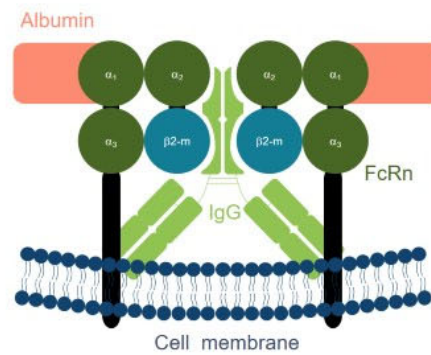


Advancing Science

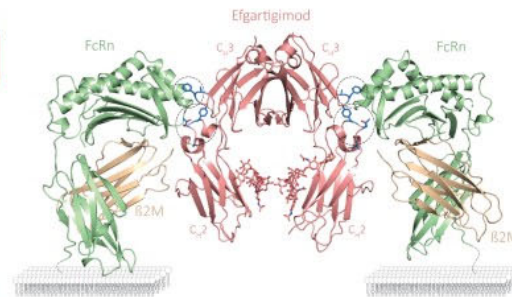


Efgartigimod Binds to FcRn in Same Formation As Endogenous IgG

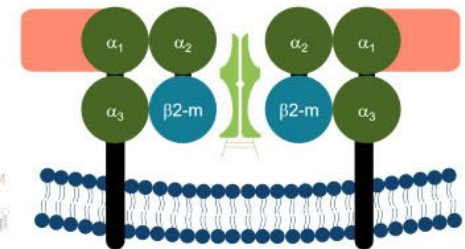
Endogenous IgG interaction



Efgartigimod



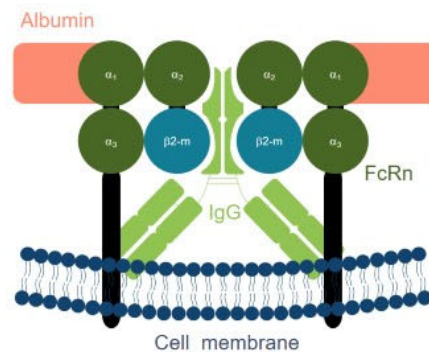
Efgartigimod interaction



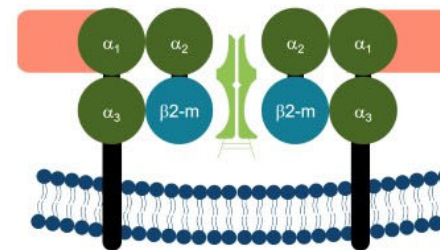
FcRn, neonatal Fc receptor; IgG, immunoglobulin G.
 1. Ulrichs P, et al. J Clin Invest. 2018;128:4372-4386; 2. Howard JF Jr, et al. Lancet Neurol. 2021;20:526-536; 3. VYVGART SmPC. Available at https://www.ema.europa.eu/en/documents/product-information/vyvgart-epar-product-information_en.pdf;
 4. Krudsen Sand KM, et al. Front Immunol. 2016;6:1-21; 5. Ward ES, et al. Front Immunol. 2022;13:892534.

Efgartigimod is Unique Among FcRn Antagonists in How it Binds

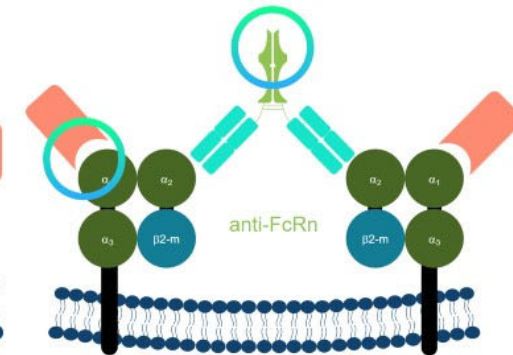
Endogenous IgG



Efgartigimod

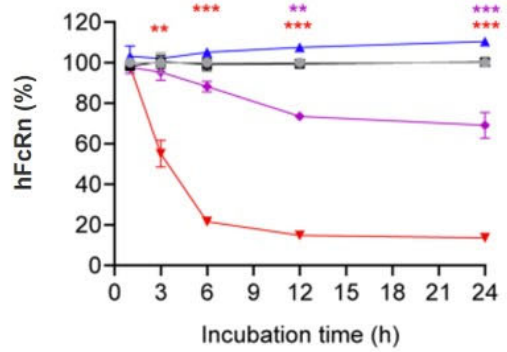


Full-length monoclonal anti-FcRn antibodies



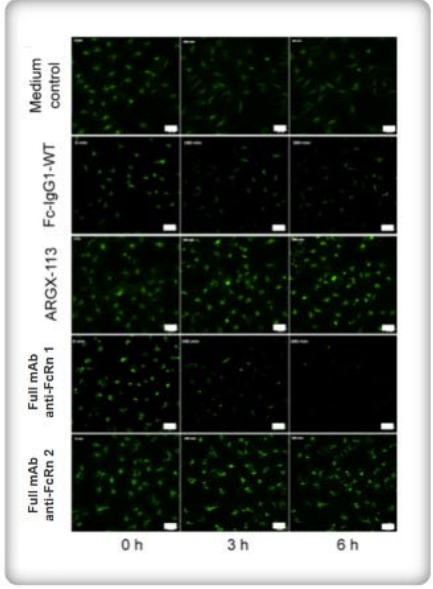
FcRn, neonatal Fc receptor; IgG, immunoglobulin G.
 1. Ulrichs P, et al. J Clin Invest. 2018;128:4372-4386; 2. Howard JF Jr, et al. Lancet Neurol. 2021;20:526-536; 3. VYVGART SmPC. Available at https://www.ema.europa.eu/en/documents/product-information/vyvgart-epar-product-information_en.pdf;
 4. Krudsen Sand KM, et al. Front Immunol. 2016;6:1-21; 5. Ward ES, et al. Front Immunol. 2022;13:892534.

Unique Binding of Efgartigimod Leads to Differentiated Intracellular FcRn Trafficking



● Medium control ▲ Full mAb anti-FcRn 1
 ■ IgG1-WT ▲ Full mAb anti-FcRn 2
 ▲ ARGX-113

Ma et al, 2024 (10.1172/jci.insight.178166)

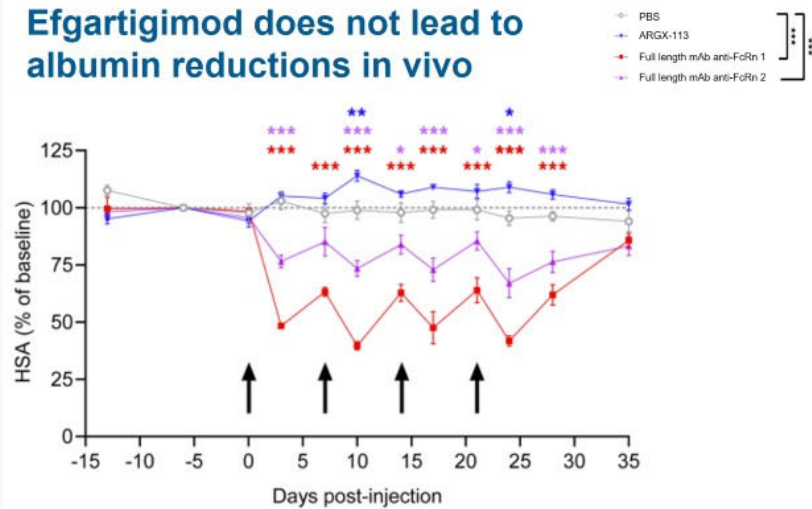


No interference of efgartigimod with albumin binding and recycling

No degradation of FcRn induced by efgartigimod

Unique Binding of Efgartigimod Positively Impacts in vivo Albumin Levels and Safety Profile

Efgartigimod does not lead to albumin reductions in vivo

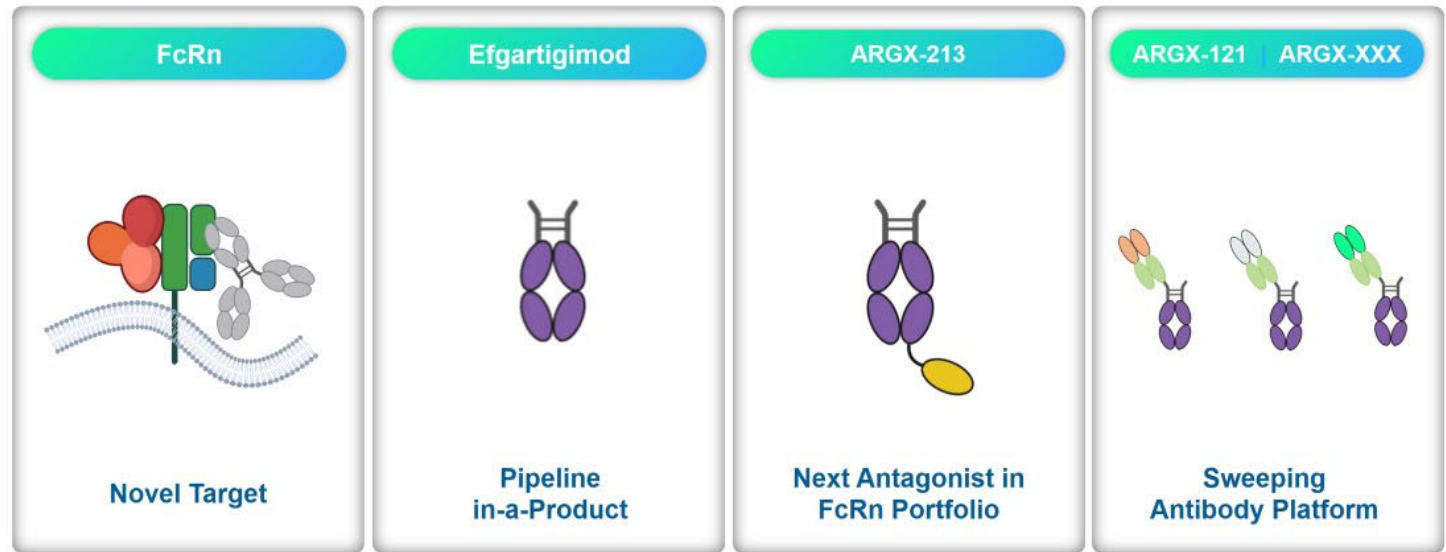


Ma et al, 2024 (10.1172/jci.insight.178166)

Efgartigimod treatment results in a favorable safety profile in the clinic

- No albumin reduction
- No edema, hyperlipidemia or muscle cramps
- No aseptic meningitis
- No clearance by anti-drug antibodies

Evolution of a Novel Target to a Novel Platform

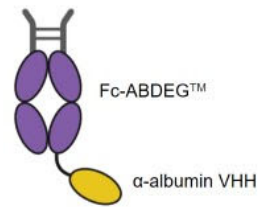


Next Wave of First-in-class Immunology Targets

Karen Silence /// Preclinical Product Development

Deep Knowledge of FcRn Biology Builds New Pipeline Candidates

Leverage Albumin to Broaden FcRn Targeting Portfolio



ARGX-213

Leveraging Efgartigimod Backbone to Build New Class of Highly Potent Sweeping Molecules

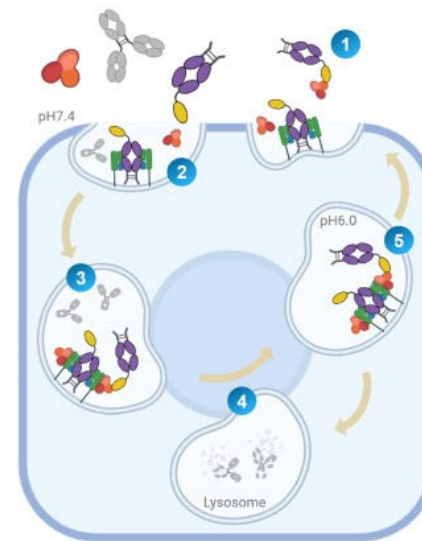
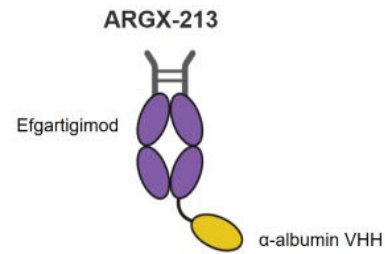
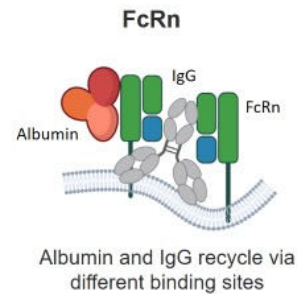


ARGX-121

ARGX-XXX

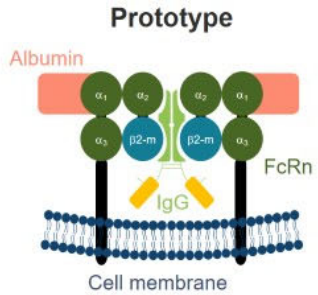
ARGX-XXX

Improving Pharmacokinetics of Efgartigimod Through Binding to Serum Albumin

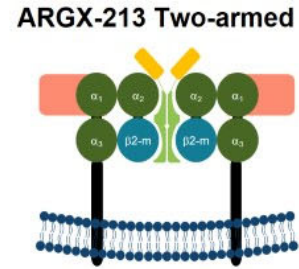
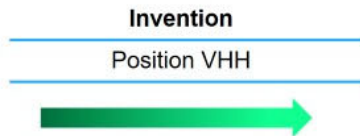


- 1 ARGX-213 adopts long albumin half-life
- 2 Pinocytosis
- 3 In endosomes, ARGX-213 prevents IgG binding to FcRn
- 4 IgG degraded in lysosomes
- 5 ARGX-213 recycles via FcRn or albumin

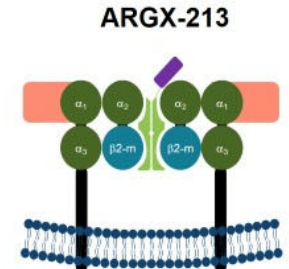
ARGX-213 is Designed For Optimal FcRn Binding and Equipped with Unique Features



- ✓ Enhanced PK
- Sustained PD
- Albumin sparing

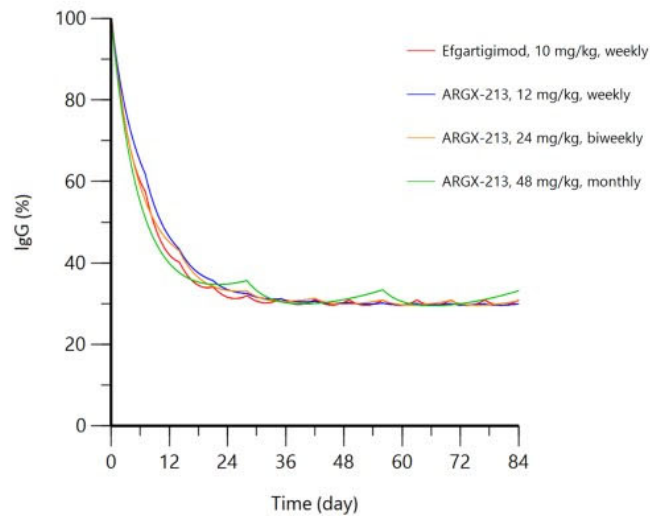


- ✓ Enhanced PK
- ✓ Sustained PD
- Albumin sparing



- ✓ Enhanced PK
- ✓ Sustained PD
- ✓ Albumin sparing

ARGX-213 Can Achieve Extended Dosing



10 mg/kg efgartigimod and 12 mg/kg ARGX-213 are equimolar doses ARGX-213 PK/PD model based on mouse and cyno data

ARGX-213 has increased half-life compared to efgartigimod resulting in prolonged PD effect

Simulations predict potential for monthly dosing

Path Forward for ARGX-213

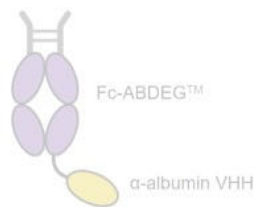
Finalize GLP
Tox Study

Submit Clinical
Trial Application
1H25

Phase 1 to Start
in 2H25

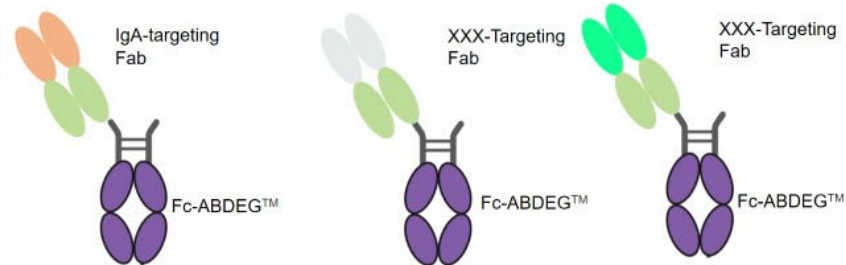
Deep Knowledge of FcRn Biology Builds New Pipeline Candidates

Leverage Albumin to Broaden FcRn Targeting Portfolio



ARGX-213

Leveraging Efgartigimod Backbone to Build New Class of Highly Potent Sweeping Molecules



ARGX-121

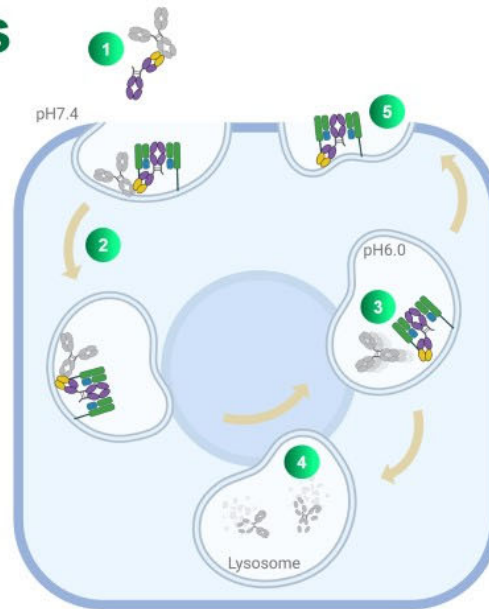
ARGX-XXX

ARGX-XXX

ARGX-121 Mode of Actions

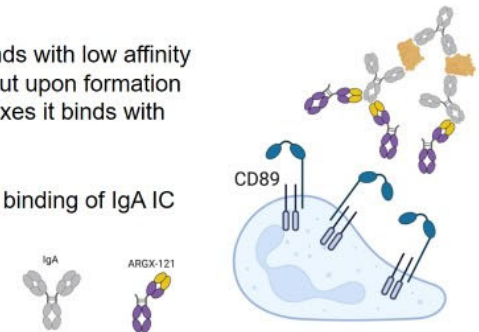
I. FcRn-mediated IgA degradation

- 1 ARGX-121 binds to IgA (1-3 mg/ml)
- 2 Enhanced endocytosis of ARGX-121 IgA complex
- 3 Complex dissociates at pH 6.0 in endosomes
- 4 IgA is degraded in lysosomes
- 5 ARGX-121 recycles through enhanced FcRn binding at pH 6.0

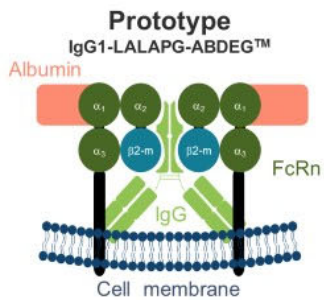


II. Blocking of IgA:CD89 mediated signalling

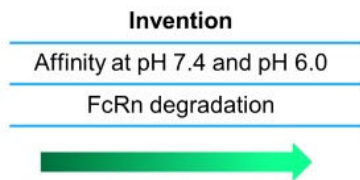
- Monomeric IgA binds with low affinity to CD89 (FcαRI) but upon formation of immune complexes it binds with high avidity
- ARGX-121 blocks binding of IgA IC to CD89



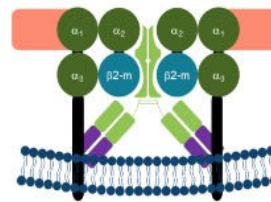
ARGX-121 Innovative Design Breakthrough



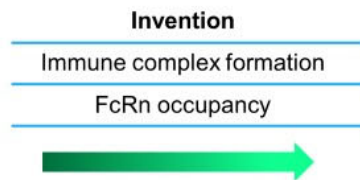
pH-dependent target binding	++
Risk for making immune complexes	+++
FcRn degradation	++
FcRn occupancy	-
IgA depletion in cyno	+



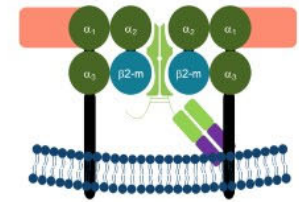
ARGX-121 Two-armed



pH-dependent target binding	+++
Risk for making immune complexes	++
FcRn degradation	+
FcRn occupancy	+
IgA depletion in cyno	++

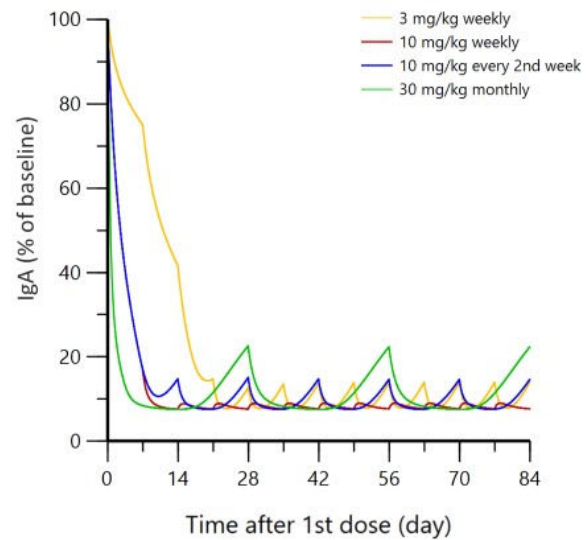


ARGX-121 One-armed



pH-dependent target binding	+++
Risk for making immune complexes	-
FcRn degradation	-
FcRn occupancy	++
IgA depletion in cyno	+++

ARGX-121 Rapidly and Drastically Impacts Circulating IgA Levels



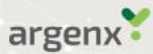
Baseline IgA levels: 2.5mg/ml
ARGX-121 PK/PD model based on mouse and cyno data

>90% IgA reduction within 1 week

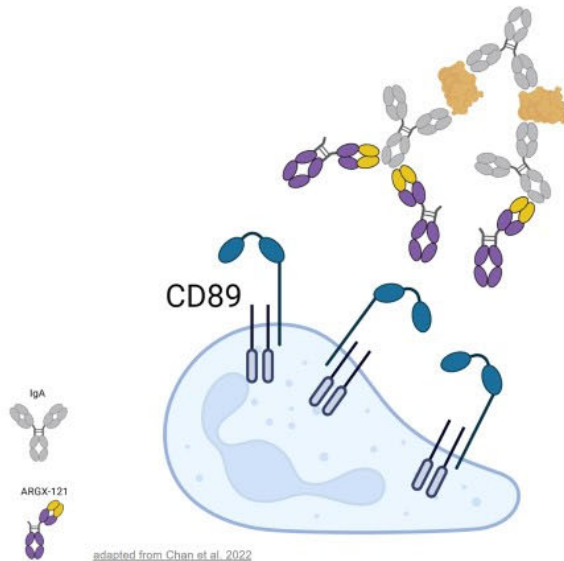
Enables flexible dosing

Broad therapeutic potential

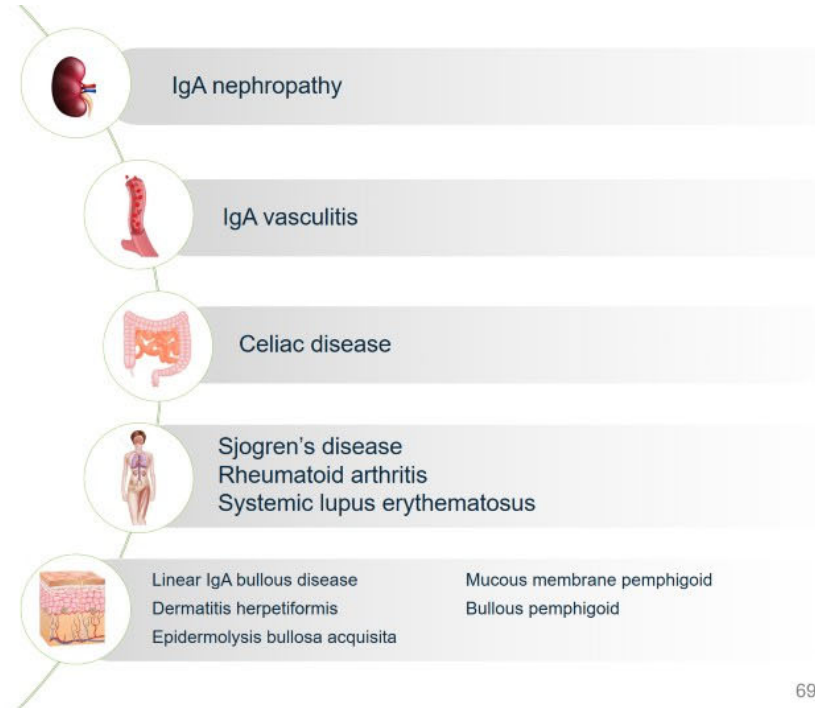
ARGX-121 Pipeline-in-a- Product Potential



IgA is Fundamental in Many Diseases



adapted from Chan et al. 2022



Path Forward for ARGX-121

Finalize GLP
Tox study

Submit Clinical
Trial Application
1H25

Phase 1 to start
in 2H25

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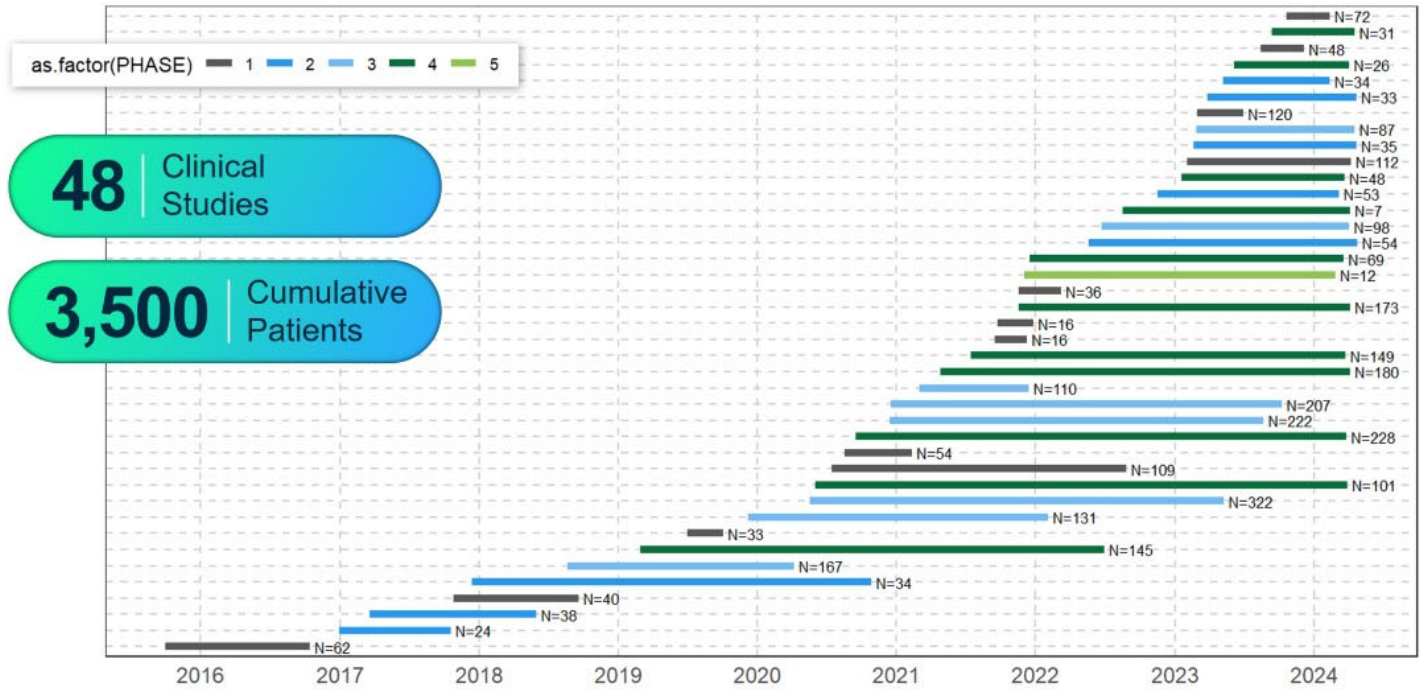
Clinical Development

Luc Truyen, M.D., PhD /// Chief Medical Officer

Clinical Development: Bridging Innovation and Unmet Patient Need



Rapidly Scaling our Clinical Footprint



Pioneering in MG to Set New Standard for Treatment

IgG lowering effect of efgartigimod

Central role of IgG in MG

Extended clinical effect of cyclical dosing

MSE >50% across studies

Highest MG-ADL and QMG response

Equivalence of SC to IV

Positive benefit risk profile

Value broadly recognized by payor bodies

Sustained responses across dosing schedules

Reduction in steroid use

Broadest safety database

Earlier treatment lines
Seronegative, Ocular

Market leader among advanced biologics

2010 - 2017

2018

2019

2020

2021

2022

2023

2024

FUTURE

Therapeutic Apheresis and Dialysis

Liu et al, 2010

JCI The Journal of Clinical Investigation

Ulrichs, 2017

Neurology

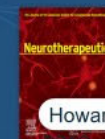
Howard et al, 2019

THE LANCET Neurology

Howard et al, 2021

frontiers in Neurology

Howard et al, 2024



Howard et al, accepted

JCI The Journal of Clinical Investigation

Ward, 2024

Applying Our Innovation Approach to Clinical Development

Innovation



Evidence Generation

Build broadest data to guide treatment decisions for patients

Co-Creation



Patient Insights

Patient engagement in trial design and execution

Execution



Speed

Bring medicines to patients as quickly as possible



Ocular and Seronegative MG



Expanding MG Leadership Across Treatment Paradigm



Evidence Generation

ADAPT/ADAPT+

Real-world data



Patient Insights

Significant need

Lack of innovation



Speed

Efficient studies

Significant underserved population

Sjögren's Disease

rhoSTUDY

argenx

Working to Reach Patients Faster



Evidence Generation

Depth of data from RHO study

Leveraging all FcRn data



Patient Insights

Endpoint selection

PRO measures



Speed

Phase 3 to start by end of 2024

Immune Mediated Myopathies (IMM)



One Study Across Multiple Myositis Subtypes



Evidence Generation

Subtype selection based on pathogenic IgG rationale



Patient Insights

Common TIS endpoint



Speed

Seamless Phase 2/3 Study with interim analysis

Multifocal Motor Neuropathy (MMN)



Pioneering First-in-Class Novel MoA



Evidence Generation

Robust PoC from ARDA

EoP2: endpoint alignment



Patient Insights

Natural history study exceeds 100 patients to date



Speed

Leveraging Ph2 and iMMersion to accelerate recruitment

CIDP is 4th Indication for Empasiprubarb



Developing a Winning Strategy in CIDP



Evidence Generation

Building on MMN data

Broadening knowledge in complement biology



Patient Insights

High medical need

Opportunity for multiple innovations



Speed

Registrational trial with interim analysis

Bringing Innovation to Patients



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Efgartigimod in Myositis

Leentje De Ceuninck, Ph.D. /// Senior Clinical Scientist



Melissa Living with Myositis

Idiopathic Inflammatory Myopathies (IIM) or Myositis

Characteristics

14 per 100,000 diagnosed

Mid-adult onset, more common in females

Increased mortality

No FDA-approved therapies across myositis subtypes

Disease Burden

Muscle weakness and Pain

Fatigue

Large impact on quality of life

Corticosteroid side effects

Myositis subtypes mediated by autoantibodies:

immune-mediated necrotizing myopathy (IMNM), Antisynthetase syndrome (ASyS) and dermatomyositis (DM)



Melissa
Living with Myositis

Myositis Specific Autoantibodies (MSA) are Associated with Different Clinical Symptoms

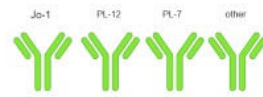
MSA target different autoantigens

IMNM



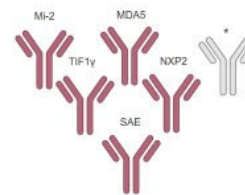
SRP: protein translation
HMGCRCR: cholesterol synthesis

ASyS



tRNA synthetases

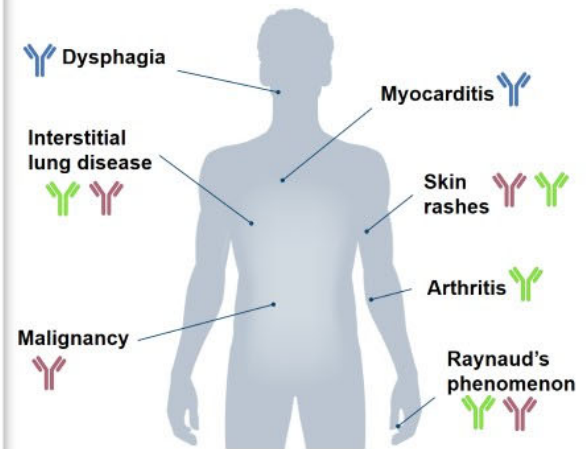
DM



Mi2: transcriptional repressor
Others: IFN regulators

* 25 – 30%: Myositis associated antibodies (MAA) or antibodies against unidentified targets

Clinical symptoms



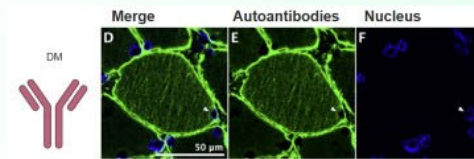
Common hallmark: proximal muscle weakness

1. McHugh J, et al. *Nat Rev Rheum.* 2018; 14(5) 2. Lundberg I, et al. *Nat Rev Dis Primers.* 2021; 7(1)

Myositis Auto-antibodies are Pathogenic

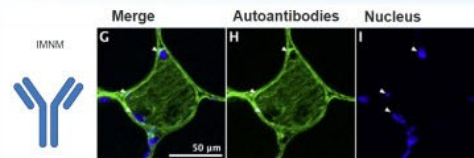
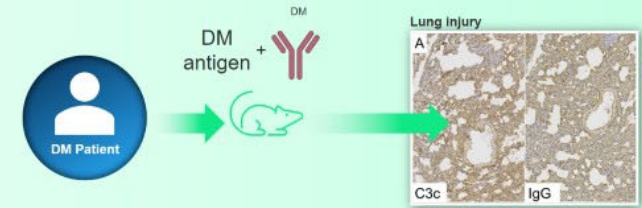


Pathogenic auto-antibodies enter muscle fibers



IFN Pathway Activation

Auto-antibodies induce IIM symptoms



Lipid Accumulation



Clinical (human data)

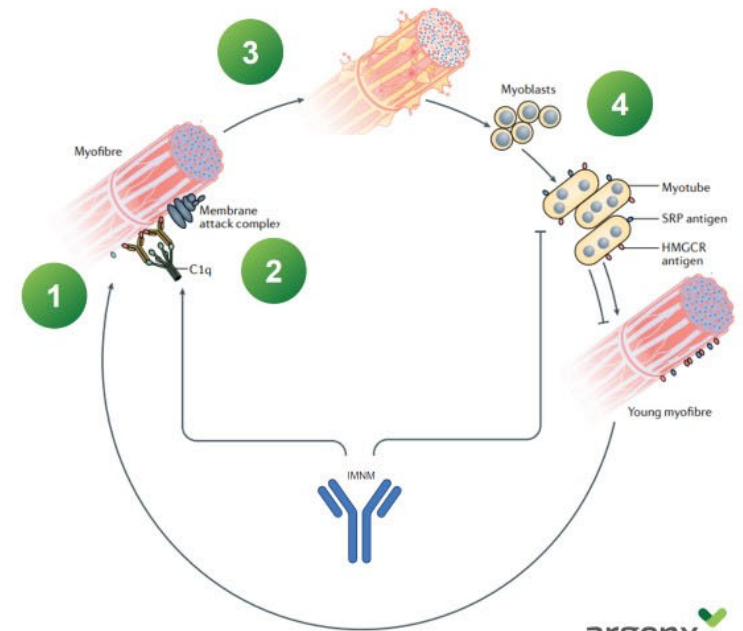
Preclinical (mouse data)

1. Pinal-Fernandez I, et al. Ann Rheum Dis. 2024 (preprint) 2. Pinal-Fernandez I, et al. Ann Rheum Dis. 2023; 82(8) 3. Bergua C, et al. Ann Rheum Dis. 2019; 78(1) 4. Zaizen Y, et al. Respir Res. 2023; 24(1)

IMNM Antibodies Trigger Muscle Damage and Impair Muscle Regeneration

Auto-antibodies:

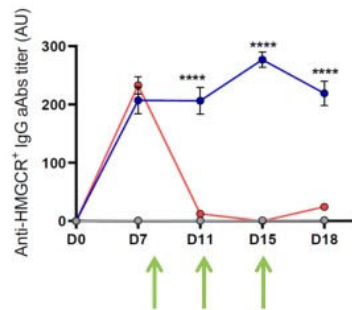
- 1 Bind muscle fiber
- 2 Activate complement
- 3 Cause necrosis
- 4 Impair muscle regeneration



1. Figure adapted from: Allenbach Y, et al Nat Rev Rheum. 2020; 16(12) 2. Bergua C, et al. Ann Rheum Dis. 2019; 78(1) 3. Arouche-Delaperche L, et al Ann Neur. 2017; 81(4)

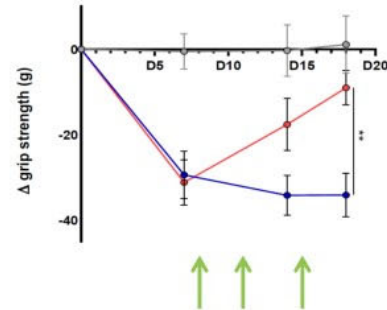
Efgartigimod Reduces IMNM Antibodies and Restores Mouse Muscle Function

Antibodies

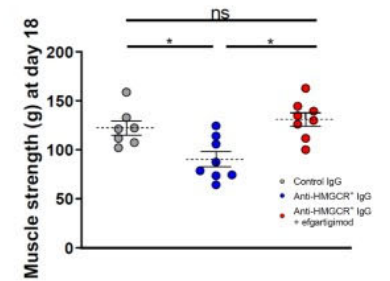


↑ Efgartigimod treatment

Grip Strength

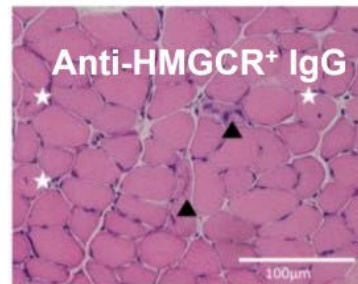


Muscle Strength

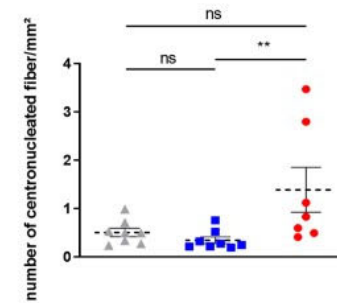
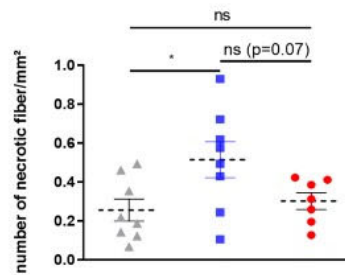


Efgartigimod Prevents Necrosis & Allows Regeneration of Muscle Fibers

Necrosis



★ Regenerating muscle fiber
▲ Necrotic fiber

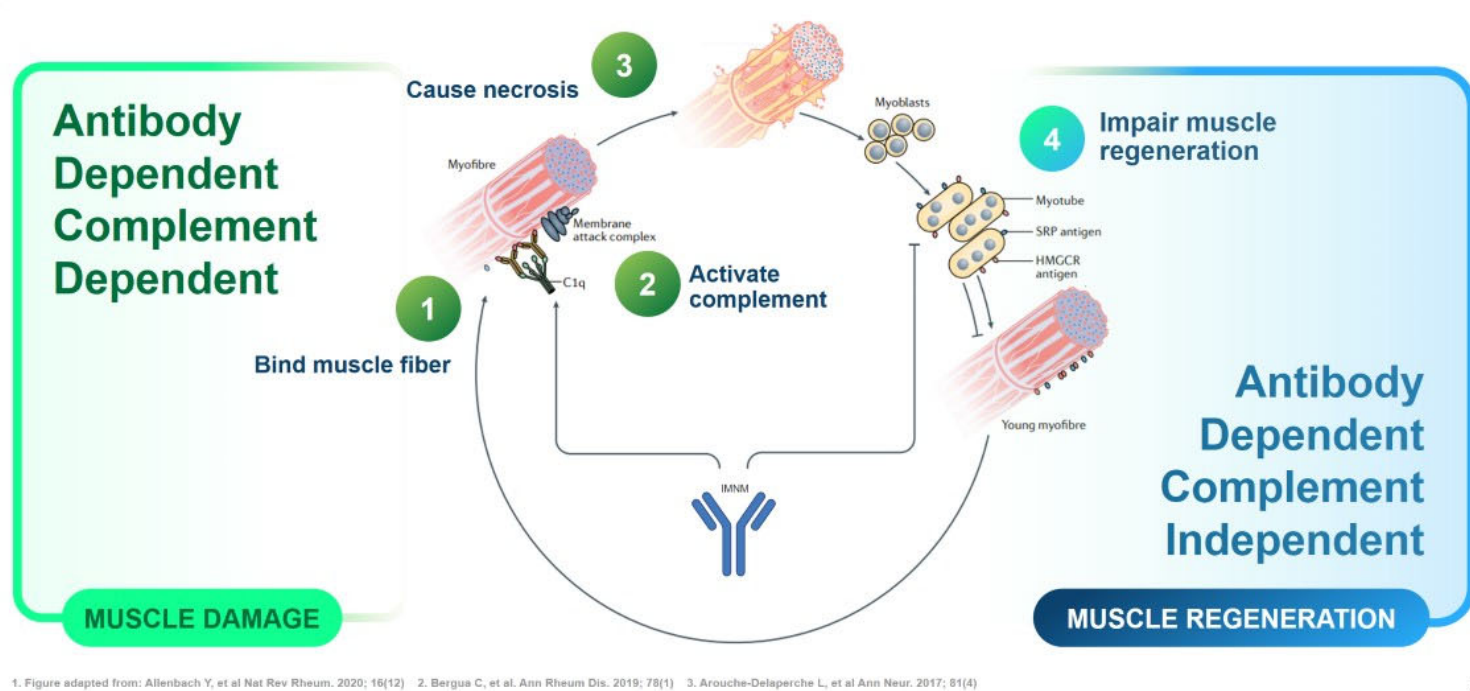
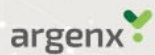


Regeneration

▲ Control IgG
■ Anti-HMGCR+ IgG
● Anti-HMGCR+ IgG + efgartigimod

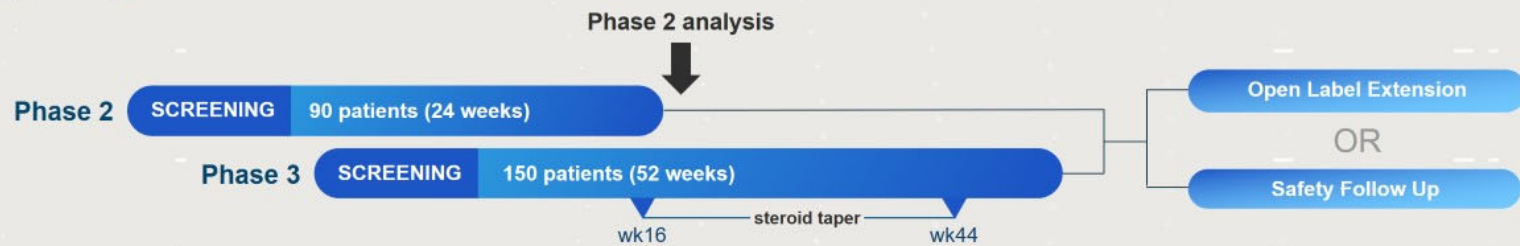
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Efgartigimod Leads to Full Regain of Muscle Function in the IMNM Mouse Model



1. Figure adapted from: Allenbach Y, et al Nat Rev Rheum. 2020; 16(12) 2. Bergua C, et al. Ann Rheum Dis. 2019; 78(1) 3. Arouche-Delaperche L, et al Ann Neurol. 2017; 81(4)

Phase 2 / Phase 3 Adaptive Basket Trials with Efgartigimod in IMNM, ASyS, DM



Adults
Active disease and muscle weakness despite stable dose of SoC

Weekly efgartigimod or PBO
+
background treatment

Phase 2 analysis
Go/NoGo per Myositis subtype
Primary endpoint: TIS

Path Forward for Myositis

**Seamless
Phase 2 / Phase 3**

**Ongoing in
IMNM, ASyS, DM**

**Phase 2 analysis
By Year End 2024**

**Go / Go No decision
on each subtype**

argenx 

Efgartigimod in Sjögren's Disease

Julie Jacobs Ph.D /// Principal Scientist



Lisa
Living with Sjögren's Disease

Sjögren's Disease

Characteristics

3 years time to diagnosis

103 per 100,000 diagnosed

55 years average age

14:1 female:male ratio

29-53% extra-glandular manifestations

Disease Burden

5-10% develop lymphoma

Decreased physical performance

Depression and **Fatigue**

Anxiety and **Pain**

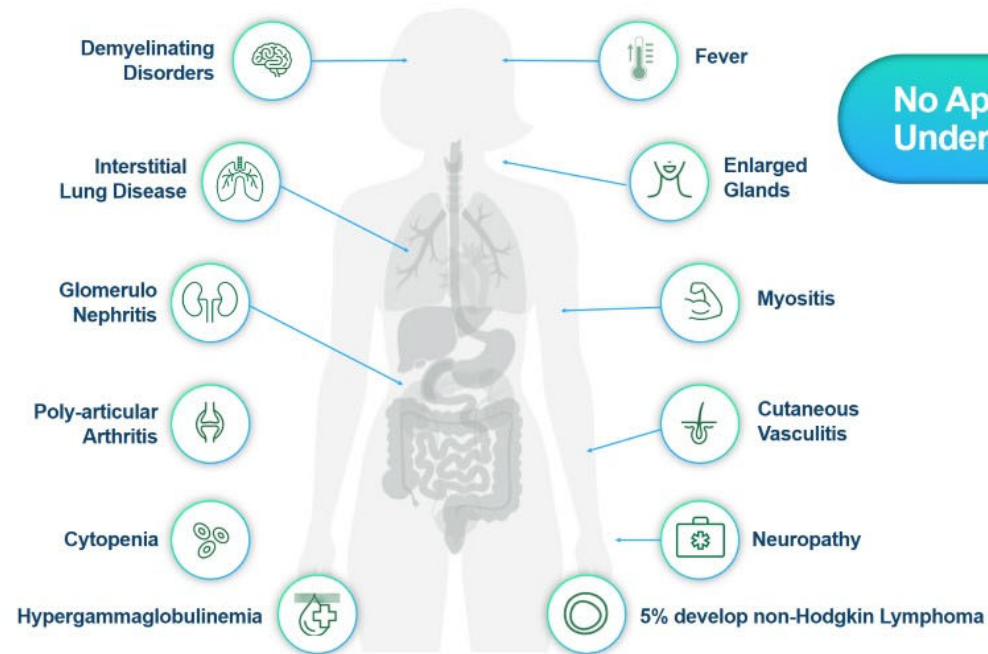
Negatively impacting **daily activities**



Lisa
Living with Sjögren's Disease

Systemic Manifestations of Sjögren's Disease

Brito-Zerón P, et al. *Nat Rev Dis Primers*. 2018; Both T, et al. *Int J Med Sci*. 2017; Negri S, et al. *Clin Exp Med*. 2022



No Approved Treatments to Target Underlying Disease

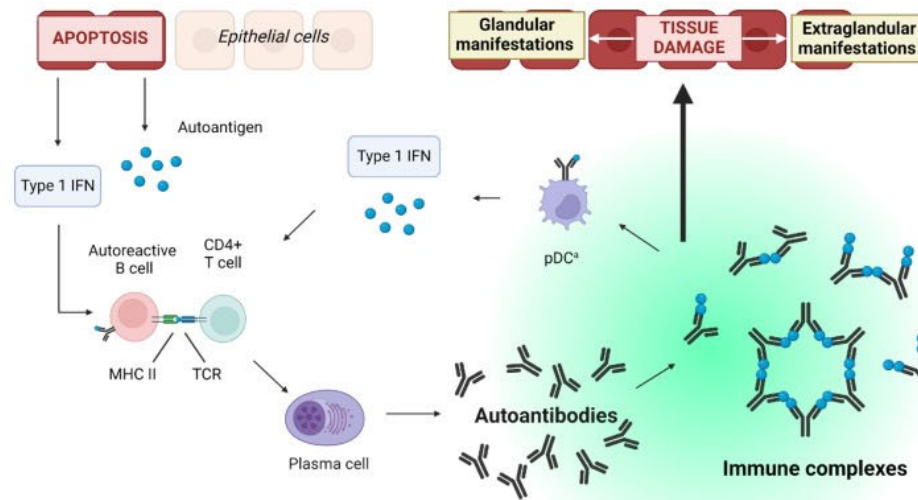
Primary Symptoms

Sicca Symptoms

Dry eye, mouth and vagina

Fatigue and Pain

Auto-antibodies are Key Players in Sjögren's Disease



Marlette X et al. 2018; Nocturne G et al. 2018; Pringle S, et al. 2019

Pathogenicity of Autoantibodies

Abnormally elevated IgG levels and presence of IgG auto-antibodies (anti-Ro/anti-La)

Auto-antibody immune complexes induce and maintain type 1 IFN signature resulting in immune-activation and tissue damage

RHO Trial: Proof-of-Concept in Sjögren's Disease



Screening Period
≤4 weeks

Key inclusion criteria

ACR/EULAR 2016 SjD diagnosed

ESSDAI ≥5

Anti-Ro+

Residual (un)stimulated salivary flow

Treatment Period
Weekly - 24 weeks

Placebo (n=9)

week 0 1 2 3 4 --- 23 24

Efgartigimod IV 10mg/kg (n=22)

Open-label Extension
48 weeks

Efgartigimod IV 10mg/kg

Weekly or biweekly dosing
depending on response

Treatment-free Follow-up Phase

Demographics and baseline characteristics

- Median age 49yo (29-70)
- ~ 5 years since diagnosis
- 68% of participants with ESSDAI ≥ 10
- Majority of patients on stable dose of hydroxychloroquine and/or low dose steroids
- 50% of patients with hypergammaglobulinemia (IgG>16 g/L)

Objectives to see consistency across measures

Primary endpoint

Proportion of responders to composite of relevant endpoints for Sjögren's disease (CRESS)

Secondary endpoints

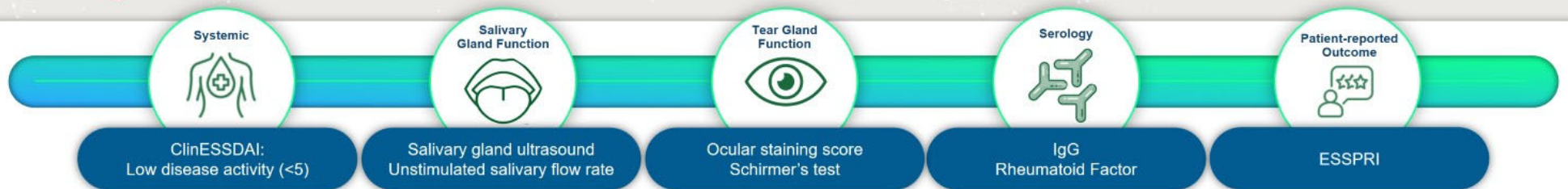
Treatment effect on

- Systemic disease (ClinESSDAI, ESSDAI)
- Patient-reported outcome (ESSPRI)
- Composite endpoint (STAR)

Biomarkers

IgG, RF, auto-antibodies, Immune complexes, IFN, histology and complement

Primary Endpoint: CRESS



OBJECTIVE:

To demonstrate more CRESS responders (at least 3 out of 5 items) at week 24 in the active arm

Limitations



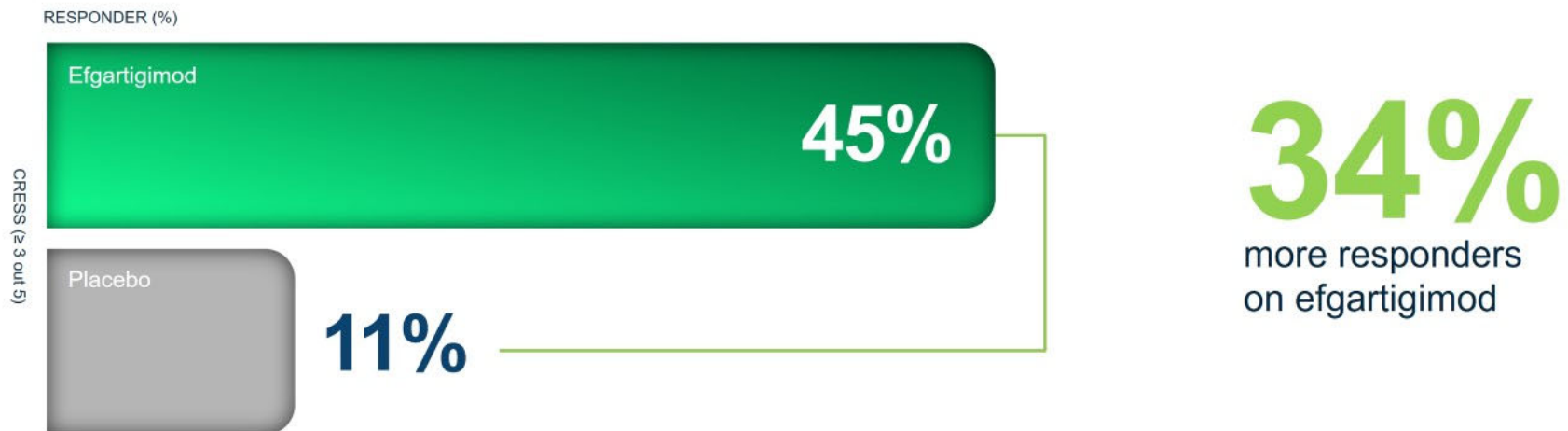
NOVEL ENDPOINT
(IN VALIDATION)

Strengths

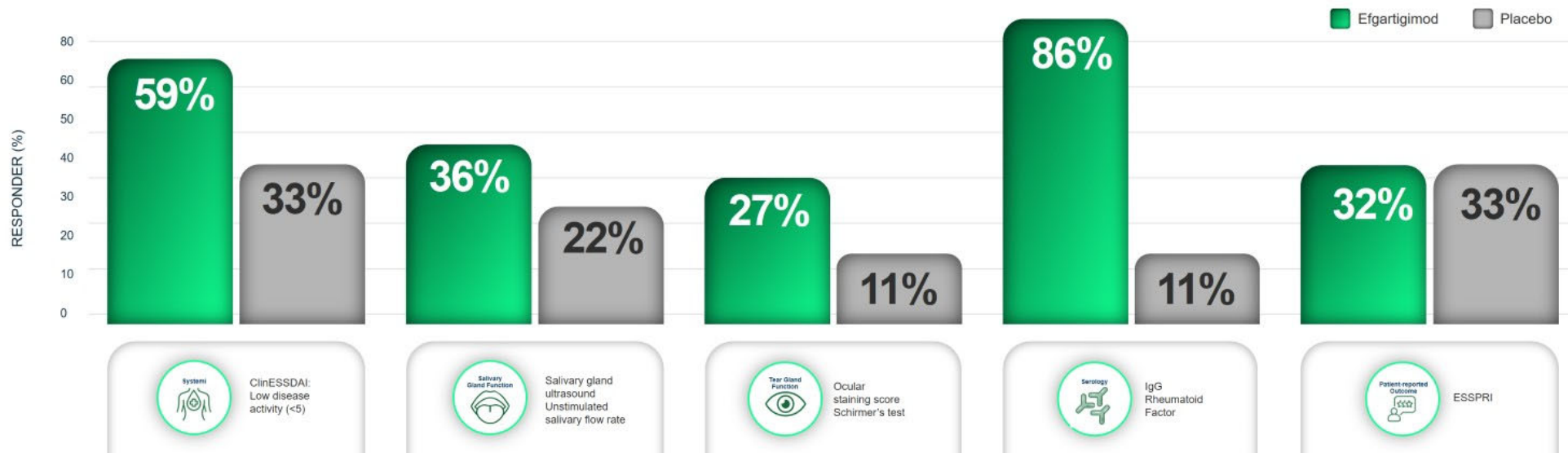


ACCOUNTS FOR
HETEROGENEOUS
DISEASE

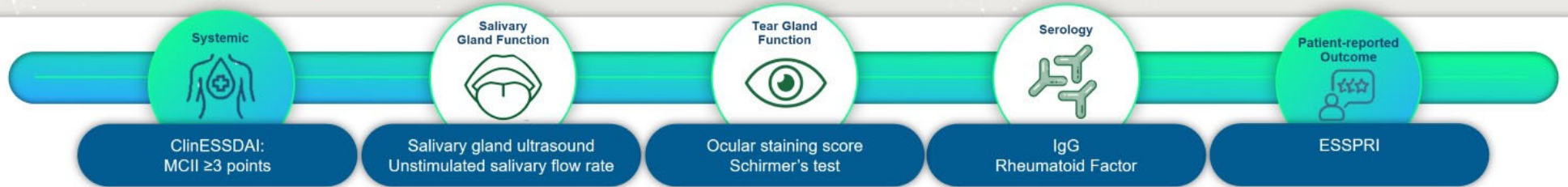
Efgartigimod Demonstrated Effect on Primary Endpoint CRESS



Observed Treatment Effect in 4 Items of CRESS



Secondary Endpoint: STAR



OBJECTIVE:

To demonstrate more STAR responders (at least 5 points) at week 24 in the active arm

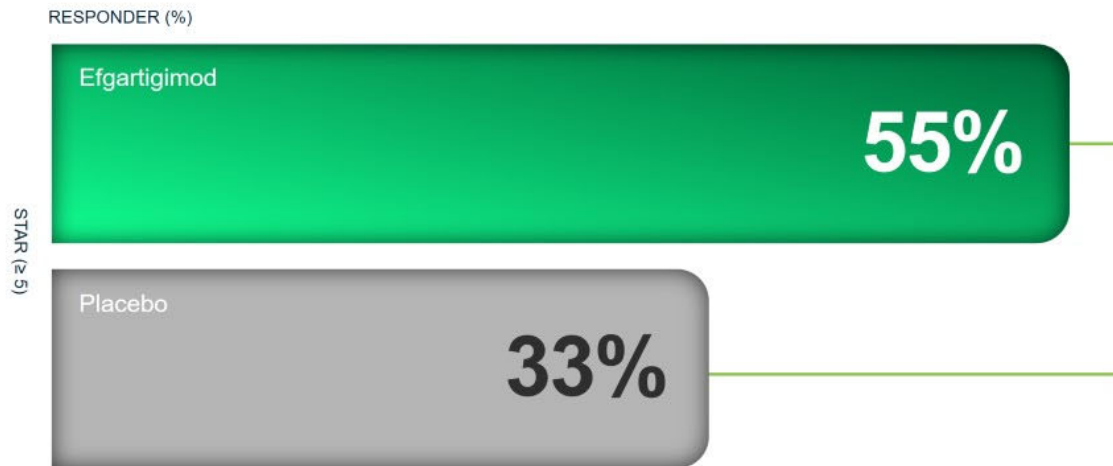
CRESS ≥ 3 out of 5

Requires response in 3 out of 5 items
Responder systemic disease: ClinESDAI < 5

STAR ≥ 5

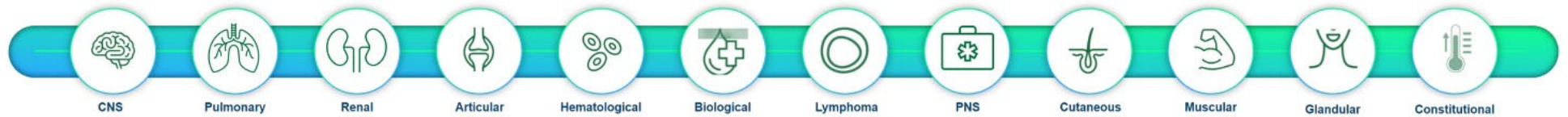
Requires response on PRO and/or systemic disease
Responder systemic disease: ClinESDAI decrease ≥ 3

Efgartigimod Demonstrated Effect on STAR



22%
more responders
on efgartigimod

Secondary Endpoint: ESSDAI



OBJECTIVE:

To demonstrate increased response rates on ESSDAI

Limitations

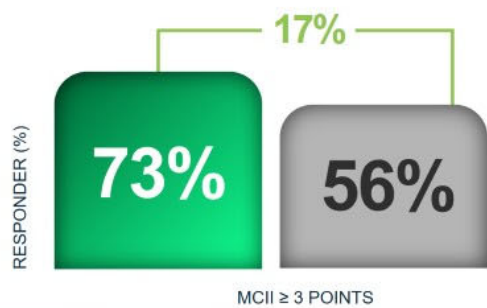
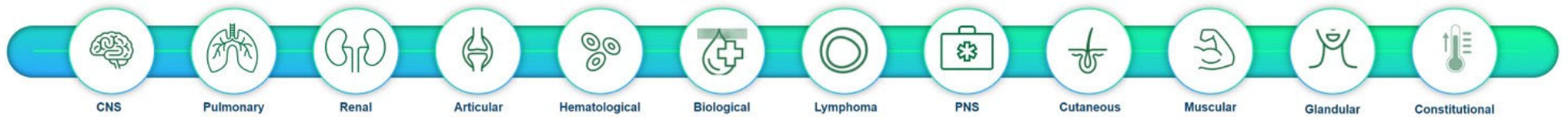
HIGH PLACEBO RESONSES

DOES NOT CAPTURE ALL DISEASE FEATURES

Strengths

ESTABLISHED ENDPOINT WITH FOCUS ON SYSTEMIC DISEASE SEVERITY

Efgartigimod Demonstrated Effect on ESSDAI

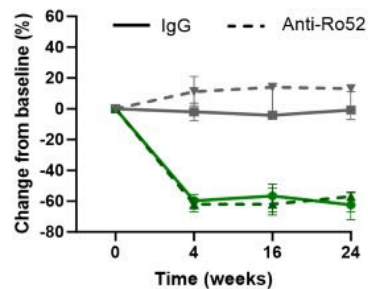


Efgartigimod
Placebo

ESSDAI

Efgartigimod Shows Potential to Break Loop of Immune Activation and Tissue Damage

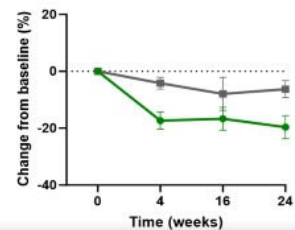
Reduction IgG and Disease-Specific Auto-Antibody



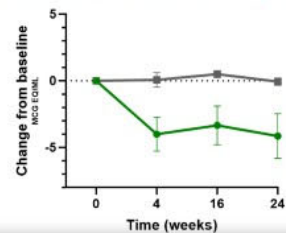
*Median ±IQR

Efgartigimod Placebo

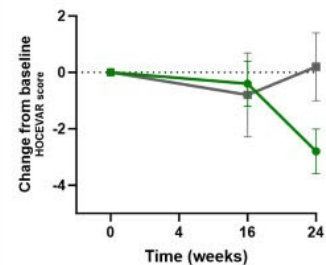
Reduction Rheumatoid Factor



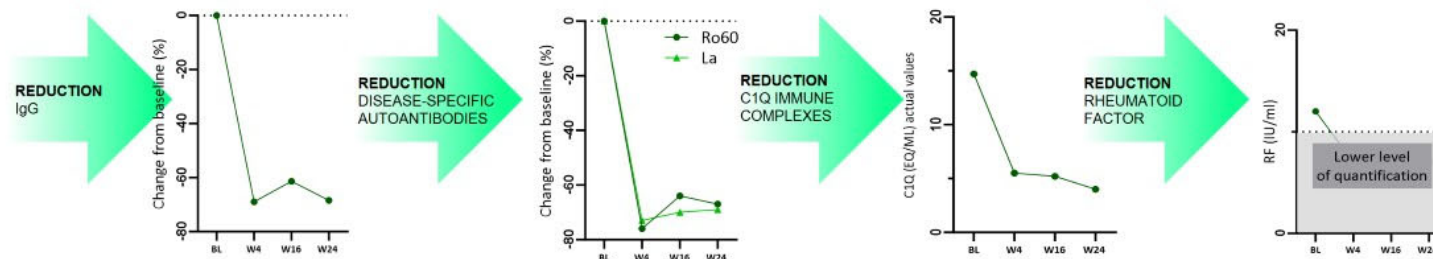
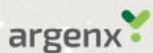
Reduction C1Q Immune Complexes



Reduction HOCEVAR Score on Salivary Gland Ultrasound



Patient Narrative Confirms Effect of FcRn Inhibition with Efgartigimod



RESPONDER ON STAR AND CRESS



Proof-of-Concept Established in Sjögren's Disease

60% IgG reductions
consistent with other
clinical trials

Reduction of auto-
antibodies, immune
complexes and
rheumatoid factor

**Increased response on
composite endpoints
(22-34%)**

Response observed in 4
out of 5 items of CRESS

Improvement over time



**Safe & well
tolerated**

**IgG Reduction and
Biomarker Data Correlate
to Clinical Benefit**

Consistency of Data Demonstrates Path Forward

rho STUDY

Phase 2 Nipocalimab Data
(DAHLIA Study)



**Justifies
Advancement To a
Phase 3 Study**

Path Forward for Sjögren's Disease

End of Phase 2
Meeting



Phase 3 to Start by
End of 2024

argenx 

Sjögren's Disease

Moderated by: Luc Truyen Ph.D., M.D. /// Chief Medical Officer

Simon Bowman, Ph.D., M.B.B.S., F.R.C.P. /// Institute of Inflammation & Aging, University of Birmingham

Julie Jacobs Ph.D. /// Principal Scientist

Sjögren's Disease

Q&A

argenx 

Empasiprubart

Inge Van de Walle /// Research Fellow



Brenda Living with MMN

Multifocal Motor Neuropathy (MMN)

Characteristics

~1.5 years to diagnosis

Progressive and often
misdiagnosed as ALS

Severe disability in **20%** of patients

IVIG only approved therapy

Disease Burden

Muscle weakness and **cramping**

Difficulty walking

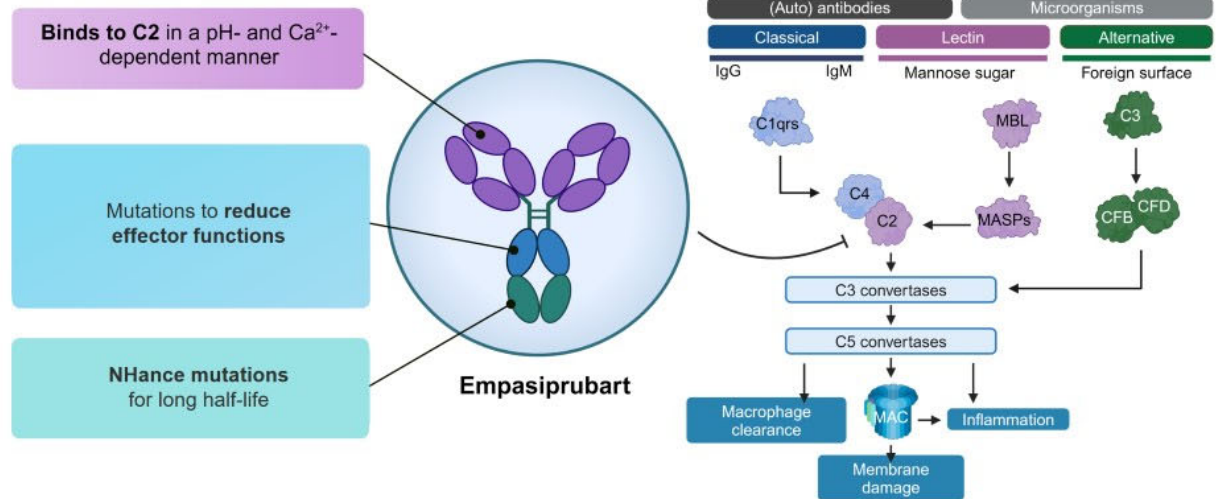
Impact on social life, activities and work

Exhaustion and **fatigue**



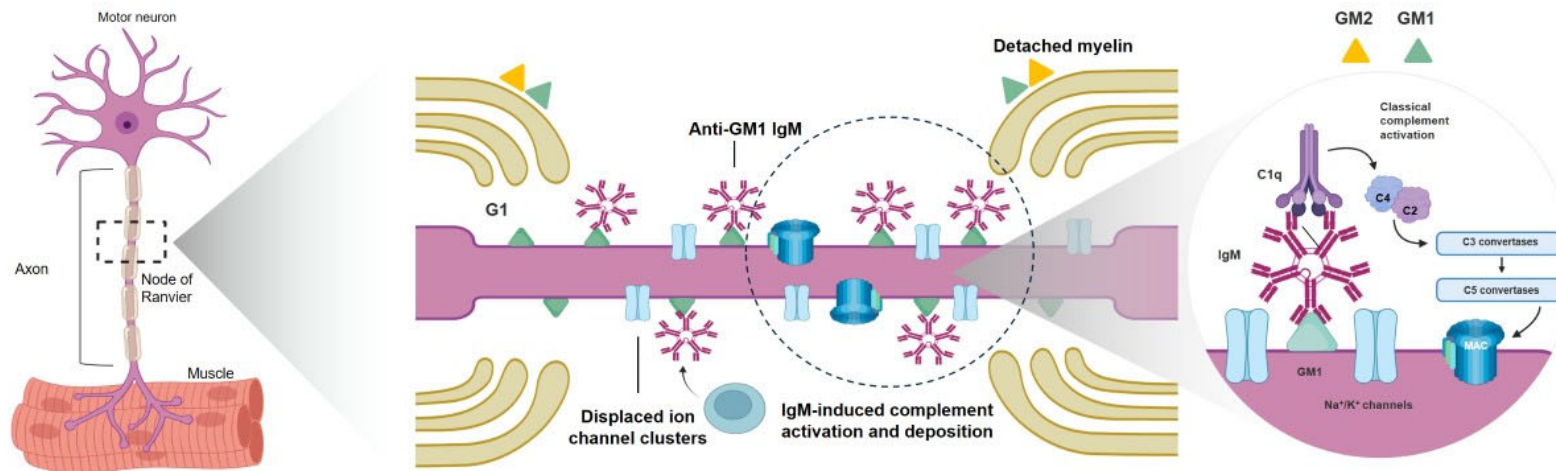
Empasiprubart

Novel C2-Specific Humanized Monoclonal Antibody With Mutations That Facilitate a Long Half-Life



Follin, neuronal Fc receptor.
 1. Maizumi K, Jorewig's Immunobiology. 8th ed. Garland Science; 2012. 2. Sarma JV, Ward PA. Cell Tissue Res. 2011;343(1):227-235. 3. Van de Walle L, et al. Clin Immunol. 2001;147(4):1420-1429.
 4. Hozaini M, et al. J Virol. 2001;75(24):12161-12168. 5. Vaccaro C, et al. Proc Natl Acad Sci. 2006;103(49):18709-18714.

Complement Activation Drives Axonal Damage in MMN

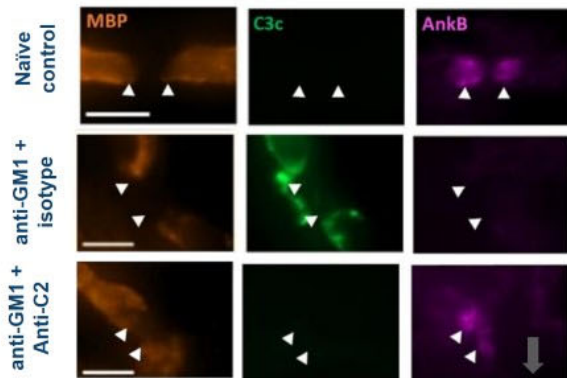


New Learning
GM2 also plays role in subset of patients

Figure created with BioRender.com, adapted from Viam L, et al. *Nat Rev Neuro*. 2011;8(1):48–58 and Sathie A, Cusick JK. In: StatPearls. Treasure Island (FL): StatPearls Publishing; 2023. <https://www.ncbi.nlm.nih.gov/books/NBK555995/>

C2 Inhibition Improves Respiratory Function in vivo

C2 inhibition reduced structural injury to Schwann cell nodal membranes

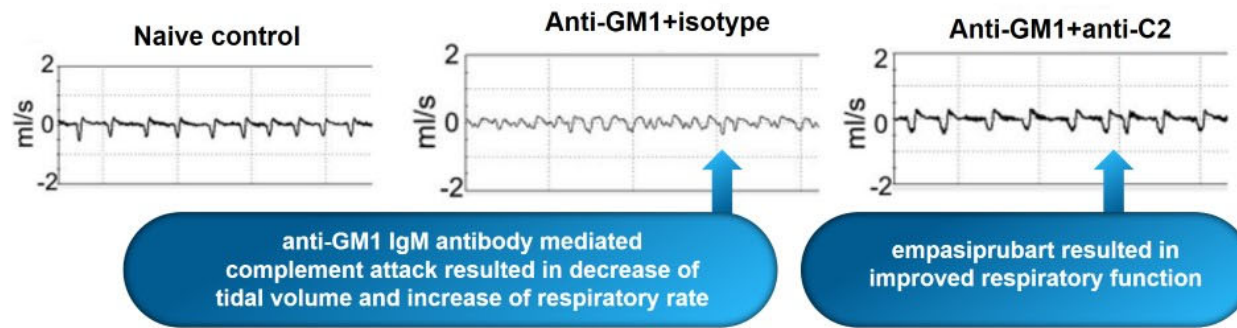


anti-GM1 antibody mediated complement attack on the Schwann cell membrane



Significant disruption at node of Ranvier (hall mark of MMN) w/o empasprubart

Empasprubart significantly reduced injury to paranodal proteins and improves respiratory function in vivo



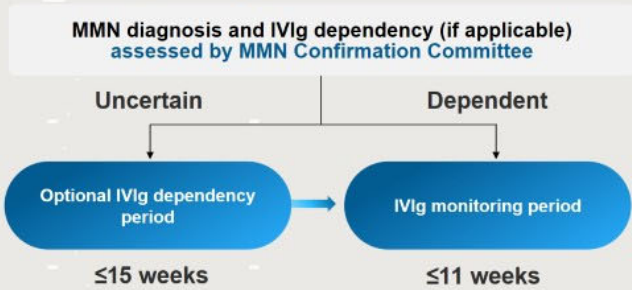
anti-GM1 IgM antibody mediated complement attack resulted in decrease of tidal volume and increase of respiratory rate

empasprubart resulted in improved respiratory function

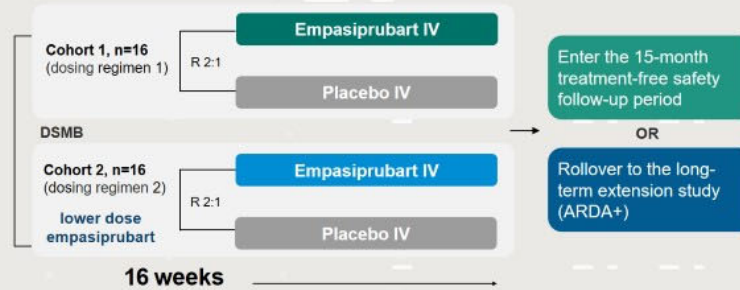
Campbell CI et al, Brain Commun. 2022 Nov 23;4(6):fcaac308

Phase 2 Trial Design

Screening (≤28 days)



Double-blinded Treatment Period



Primary endpoint Safety outcomes based on AE monitoring and other safety assessments (clinical laboratory tests)

Secondary and additional endpoints

- Time to first retreatment with IVIg
- Evaluation of efficacy measures
- Evaluation of productivity, treatment satisfaction and QoL measures
- Evaluation of PK, PD, and immunogenicity



EFNS, European Federation of the Neurology Societies; IV, intravenous; IVIg, intravenous immunoglobulin; MCC, MMN Confirmation Committee; MMN, multifocal motor neuropathy; PNS, Peripheral Nerve Society.
 *IVIg dependency parameters are summarized in the key inclusion criteria, full details provided at <https://www.clinicaltrials.gov/study/NCT05225675>. **The length of the monitoring period will depend on an individual's IVIg dose frequency: dosed every 2 weeks - up to 35 days monitoring, dosed every 3 weeks - 48 days monitoring, dosed every 4 weeks - 63 days monitoring, dosed every 5 weeks - 77 days monitoring. †Double blinded treatment period will begin 7 days after final IVIg administration. Participants will be retreated with IVIg if there is a clinically meaningful deterioration in muscle strength and/or motor function.
 †. ClinicalTrials.gov identifier: NCT05225675. Updated July 25, 2023. Accessed April 2024. <https://www.clinicaltrials.gov/study/NCT05225675>. 2. van der Pol, WL, et al. Poster presented at NMSG Annual Scientific Meeting, September 22-24, 2023, Orlando, FL.

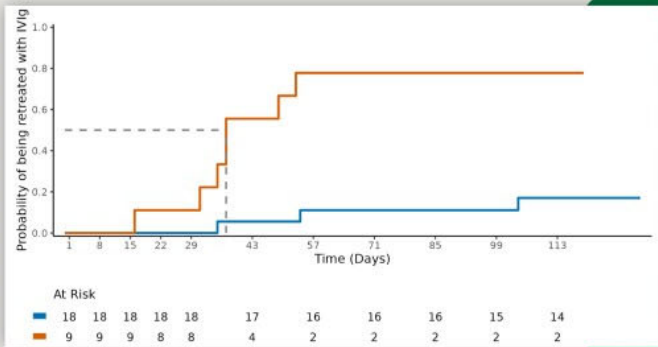
ARDA Study Results

Jeff Guptill /// Neuromuscular Franchise Lead Clinical Development

Empasiprubarb Reduced Risk of IVIg Retreatment

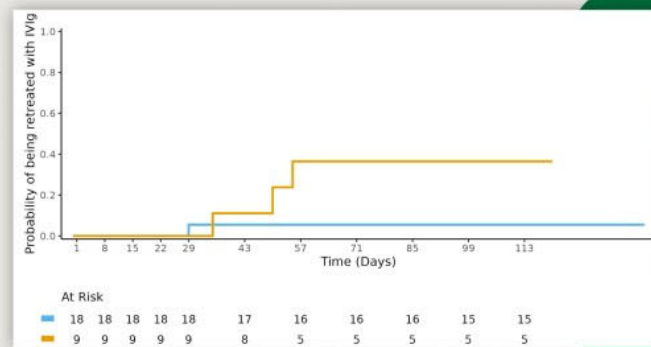


Cohort 1



Reduced risk of IVIg retreatment by **91%**

Cohort 2



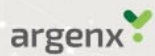
Reduced risk of IVIg retreatment by **84%**



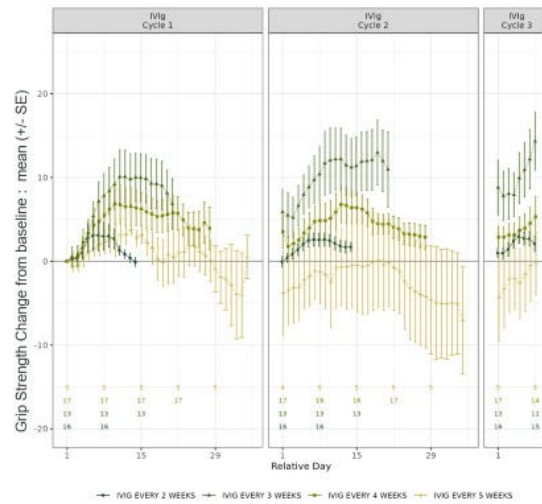
CI, confidence interval; DBTP, double-blind treatment period; IVIg, intravenous immunoglobulin.
*Time to first treatment with IVIg is defined as the time from last IVIg administration before randomization (including unscheduled visits) up to the first IVIg retreatment during the DBTP.

■ Empasiprubarb ■ Placebo

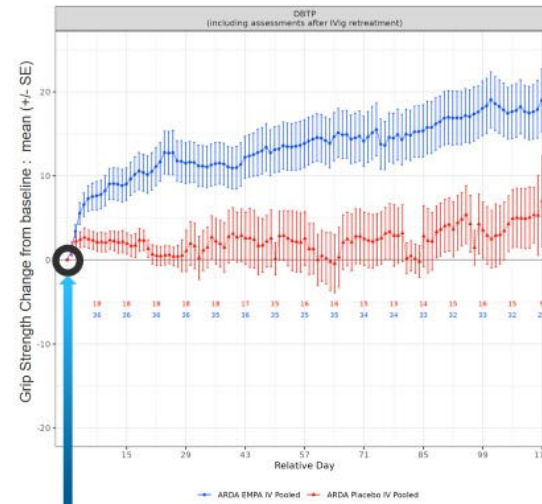
Empasiprubart Improved Grip Strength in Both Hands



IVIg Treatment → Clear Fluctuating Effect



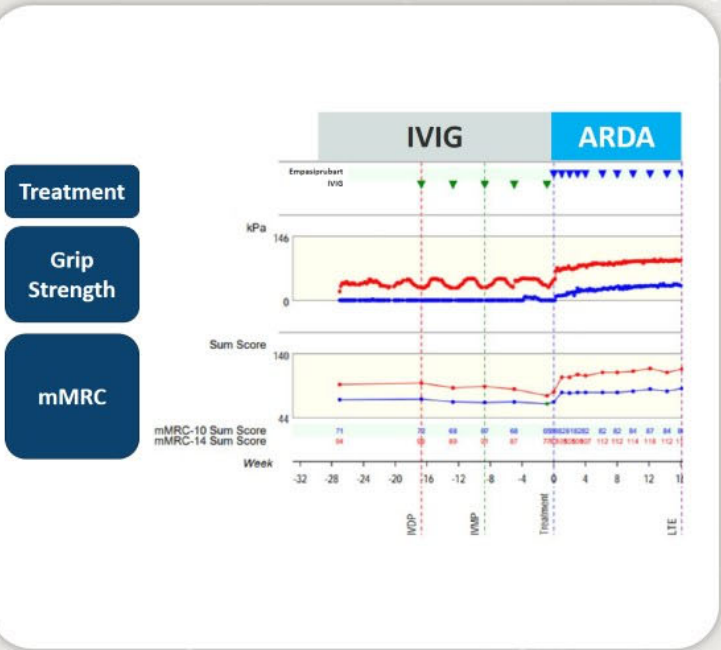
Grip Strength



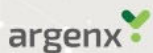
Patients at IVIG best



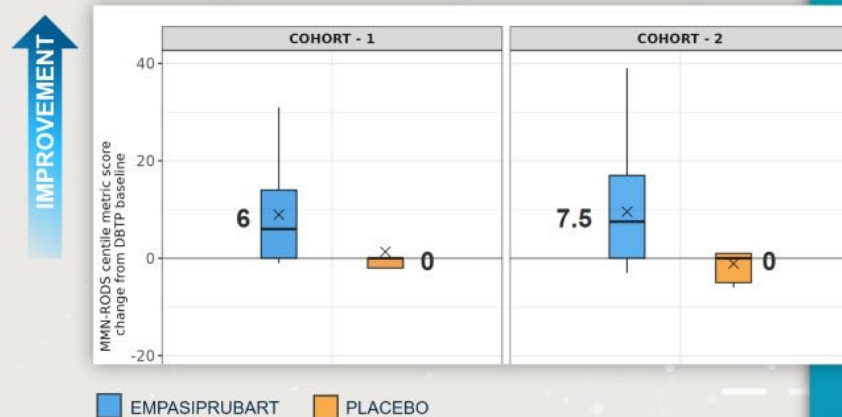
ARDA Participant Journey



Empasiprubart Improved Disease-Specific Activity Limitations Indicating Improvement in Functionality Levels



Change From Baseline of MMN-RODS Score by Treatment Group at Last Assessment During Treatment Period



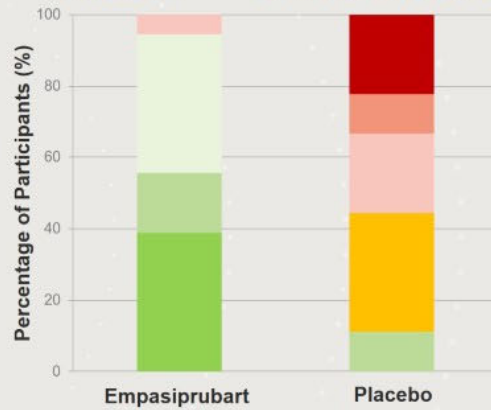
Are you able to:

- Read a book?
- Make a telephone call?
- Eat?
- Open and close a door?
- Dress your upper body?
- Brush your teeth?
- Drink out of mug/glass?
- Turn a key in a lock?
- Use knife/fork (spoon)?
- Clean after toilet?
- Fill in a form/write?
- Zip your trousers?
- Get money from cash machine?
- Do your own cooking?
- Pick up small object?
- Work on a computer?
- Do the bed?
- Fold laundry?
- Throw an object (e.g., ball)?
- Slice vegetables?
- Peel an apple/orange?
- Handle small objects (e.g., coin)?
- Tie your laces?
- Clip your finger nails?
- Button your shirt/blouse?

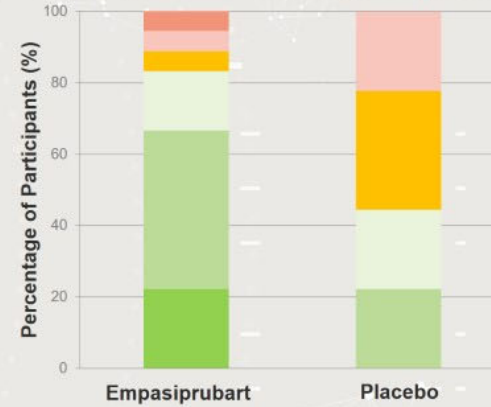
Empasiprubart Treated Patients Feel Better than their Best on MIG

How much has your condition (MMN) changed as compared to the time you received the first treatment in this trial?

Cohort 1 — 94.4% improved



Cohort 2 — 83.3% improved



- Very much improved
- Much improved
- Minimally improved
- No change
- Minimally worse
- Much worse
- Very much worse

Consistent improvement observed for each dose of empasiprubart

Path Forward for MMN

End of Phase 2
Meeting 3Q 2024

Phase 3 to Start
in 4Q 2024

IMMERSION STUDY



Trials Ongoing with Empasiprubart

varvara
Delayed Graft Function Study

DGF

Delayed Graft Function

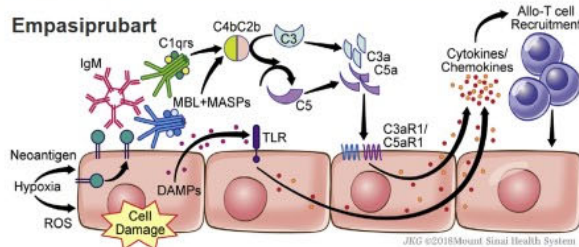
empacific
Dermatomyositis Study

DM

Dermatomyositis

Empasiprubart in Delayed Graft Function After Kidney Transplant

Biological Rationale



- Complement activation due to damaged endothelial
- Clear involvement of Classical and Lectin Pathways
- Blocking C2 improved kidney function

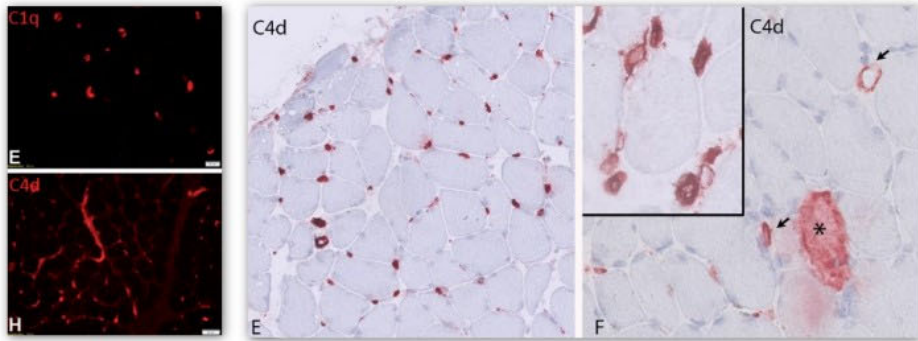
Disease Characteristics

- **40%** occurrence among cold kidney transplants
- Ischemia reperfusion injury (IRI) contributor to DGF
- Short and long-term graft negative effects
- **No current FDA-approved therapies**

Timeline Phase 2 ongoing

Empasiprubarb in Dermatomyositis

Complement Deposition in Biopsies



Disease Characteristics

- Multifactorial, idiopathic inflammatory myopathy
- Progressive and symmetric proximal muscle weakness
- IVIg is only approved treatment

Timeline Phase 2 study planned to start this year

© Basila et al. 1994; Pyltel. Appl Immunohistochem Mol Morphol. 2014 Oct 22(9):666-704; b. Campi et al. 2007; c. Lahora et al. Brain. 2016 Jul 13(9):171-180; d. Emile-Smith and Engel. 1992; e. Dalakas. 2015

2. Dalakas et al. Lancet. 2003 Sep 20; 362(9388):971-82. 3. Dalakas. Nat Clin Pract Rheumatol. 2005 Apr 2(4):219-27. Aggarwal R, Charles-Schoeman C, Scherstel J, Cimachuk MM, Beckmann I, Lovino T Medicine (Baltimore). 2021 Jan 8; 100(1):e29677

Our Next Pipeline-in-a-Product Asset

arda
Rituximab in Relapsing and Remitting Multiple Sclerosis Study

MMN

varvara
Delayed Graft Function Study

DGF

empacific
Dermatomyositis Study

DM

CIDP

...

argenx 

MMN Disease

Moderated by: Luc Truyen Ph.D., M.D. /// Chief Medical Officer

Patrick Kwon, M.D. /// Clinical Associate Professor, Neurology, New York University Grossman School of Medicine

Jeff Guptill, M.D. /// Neuromuscular Franchise Lead Clinical Development

MMN Disease

argenx 

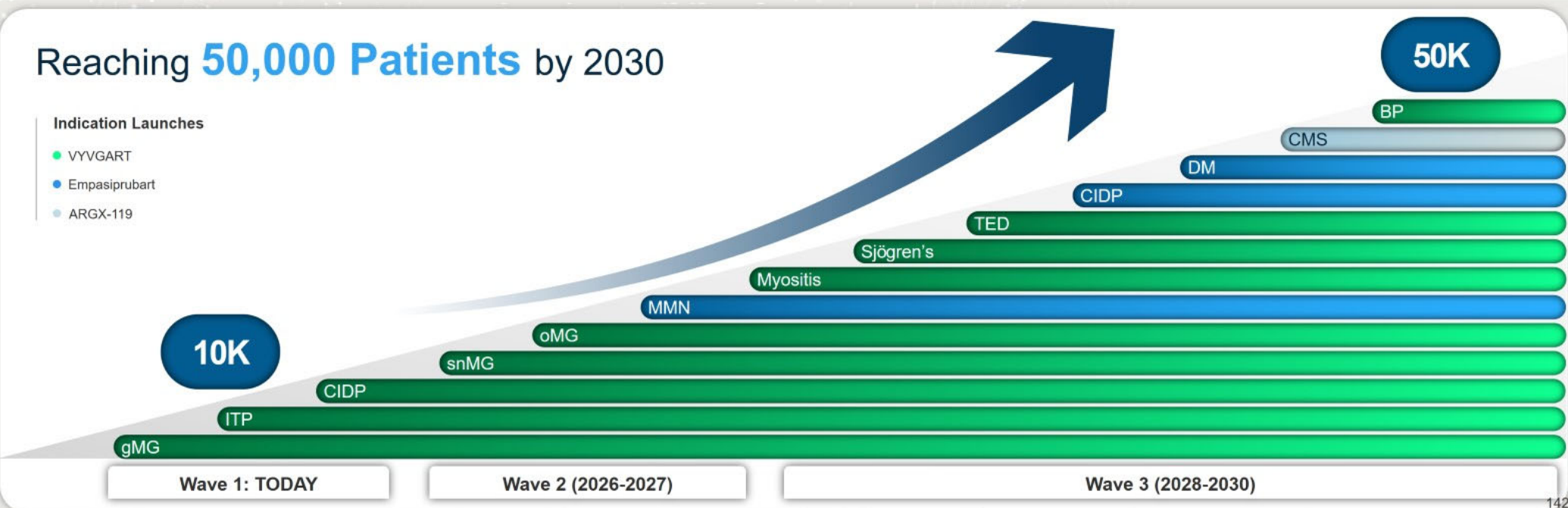
Sustainable Commercial Engine

Karen Massey /// Chief Operating Officer

Reaching 50,000 Patients by 2030

Indication Launches

- VYVGART
- Empasiprubart
- ARGX-119



MG Launch Set Standard on Commercial Excellence

Innovation



Evidence Generation

~50% MSE demonstrated across trials, sustained response across 9 cycles

Meaningful **steroid reduction**



Co-Creation



Empowering Patients

Direct to Consumer engagement

92% Brand awareness

My VYVGART[®] Path

Execution



Speed

>2,700 US prescribers

#1 among advanced biologics

Approved across **3 continents** within one calendar year

>\$1BN in year 2 of launch

9 quarters of growth

>10,000 patients globally

Future Drivers of Growth in MG

Innovation



Evidence Generation



Co-Creation



Empowering Patients

Product Presentations



PFS

Autoinjector



Execution

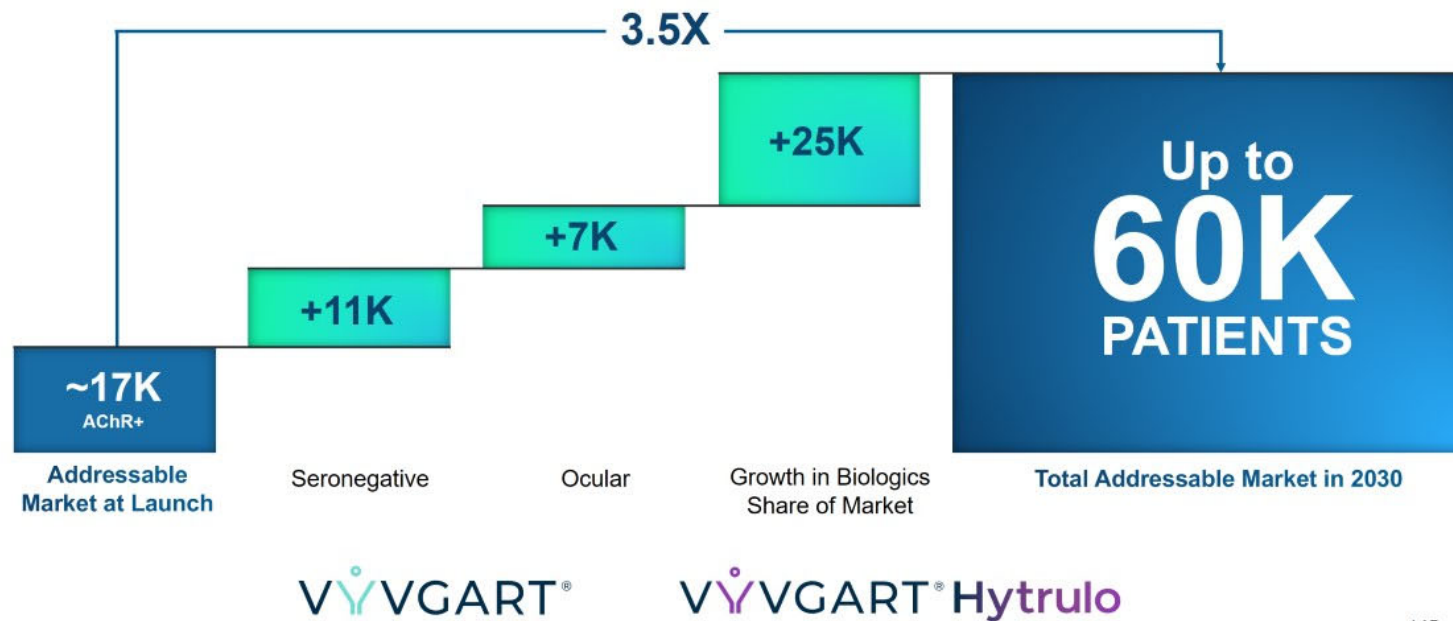


Speed

Global Expansion



Expanding MG Opportunity



Innovation Builds Markets

MG Market Dynamics are Similar to MS

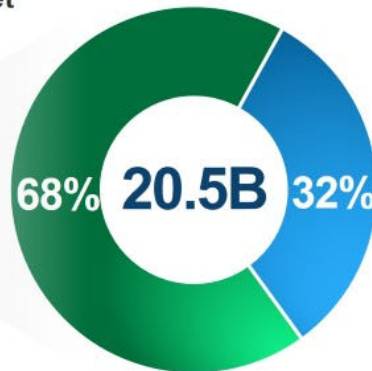


Multiple Sclerosis Market

- Advanced Treatments
- Legacy Treatments



>6X growth in advanced therapy share of market



2012

2023

Over 10+ Year Period... Market Growth was Driven by

Novel mechanisms of action

Multiple launched assets

~15% prevalence increase

More Innovation = More Prescribers, Better Outcomes for More Patients

Early Excitement in CIDP

Rapid Execution



25% of key target physicians
reached in 14 days

First payor policies in principle

Early Adoption

Prescriber breadth and depth
~20% are new to VYVGART

My VYVGART® Path

First patients on treatment

MMN: Opportunity to Build a Market

MMN Today

10K
PATIENTS

More Innovation =
More Prescribers,
Better Outcomes
For Patients

The argenx advantage

Innovation



Natural History Study to understand real-world experience

Co-creation



Engagement with patients

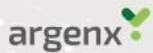


Execution



Deep existing neurology relationships

TED and Sjögren's Disease Represent MG Sized Opportunities



Path to Transforming Outcomes with Differentiated Treatments

Thyroid Eye Disease

100K Prevalence

~80K
Chronic
Patients



Limitations of existing therapies, considering safety and efficacy

Sjögren's Disease

330K Prevalence

~100K
Moderate to
Severe



No currently approved treatments to target underlying disease

*U.S. prevalence numbers, argenx market research

Vision 2030

5

New Molecules
in Phase 3

10

Labeled
Indications

50k

Patients on
Treatment

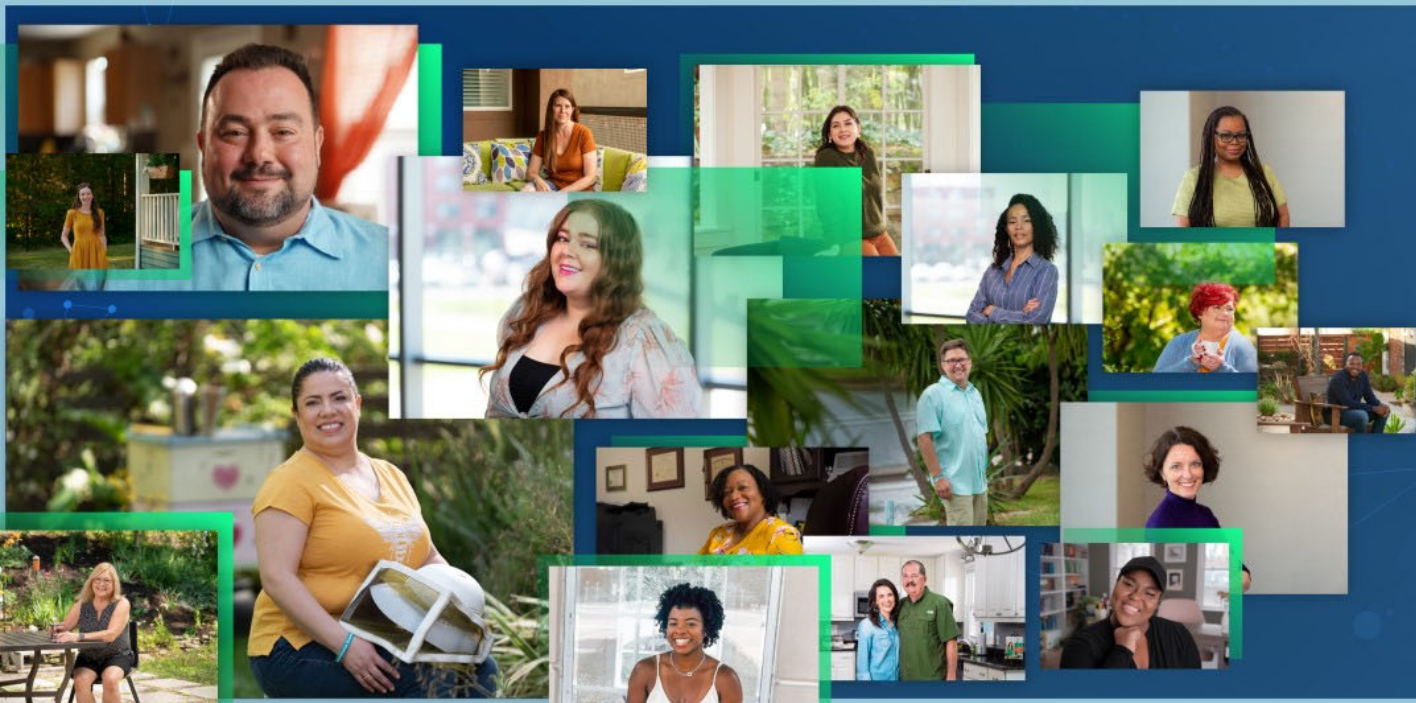
COMMITMENT TO OUR TRANSFORMATION MISSION

Continuous Pipeline of Innovation

Leadership in FcRn

Disciplined Scaling

argenx 



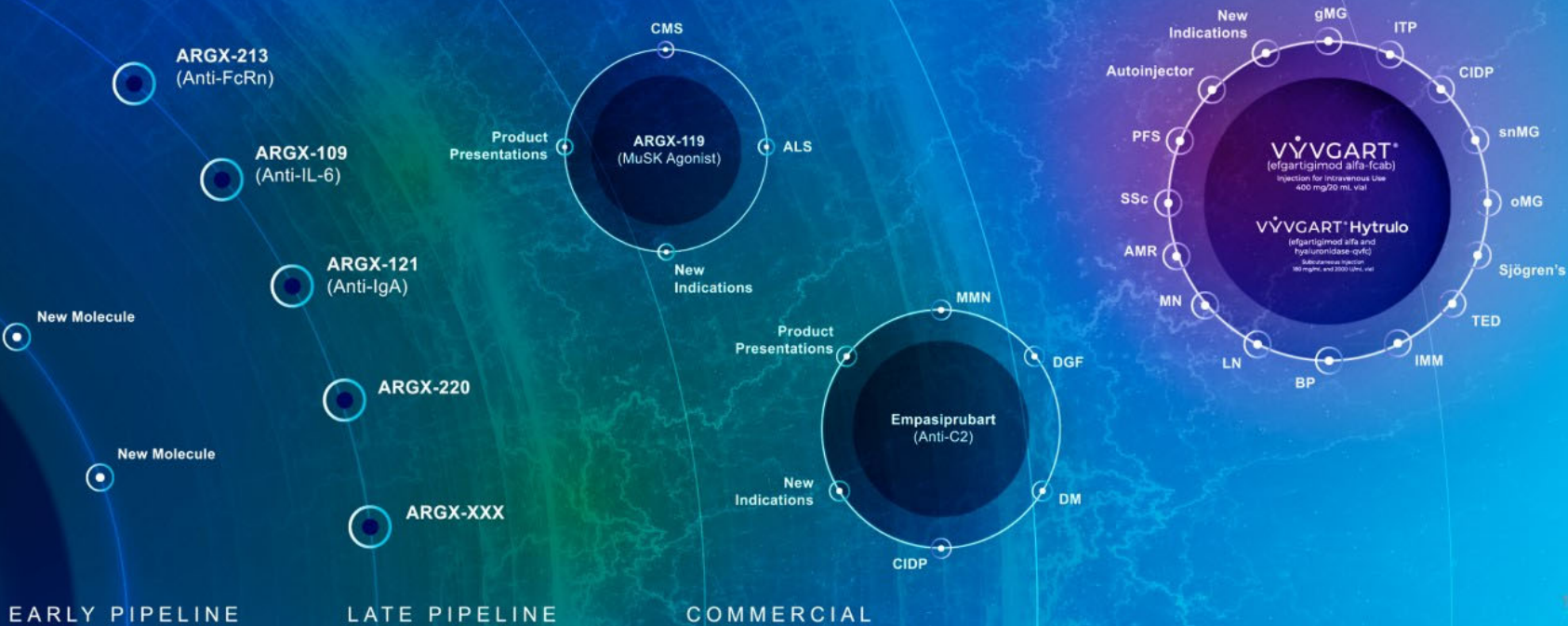
Innovation has no meaning unless it reaches patients and provides real benefit

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Q&A

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Our Horizons



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