

# 2019 Half Year Results

**argenx**

**Tim Van Hauwermeiren, CEO**

**Keith Woods, COO**

**Eric Castaldi, CFO**



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## Forward-Looking Statements

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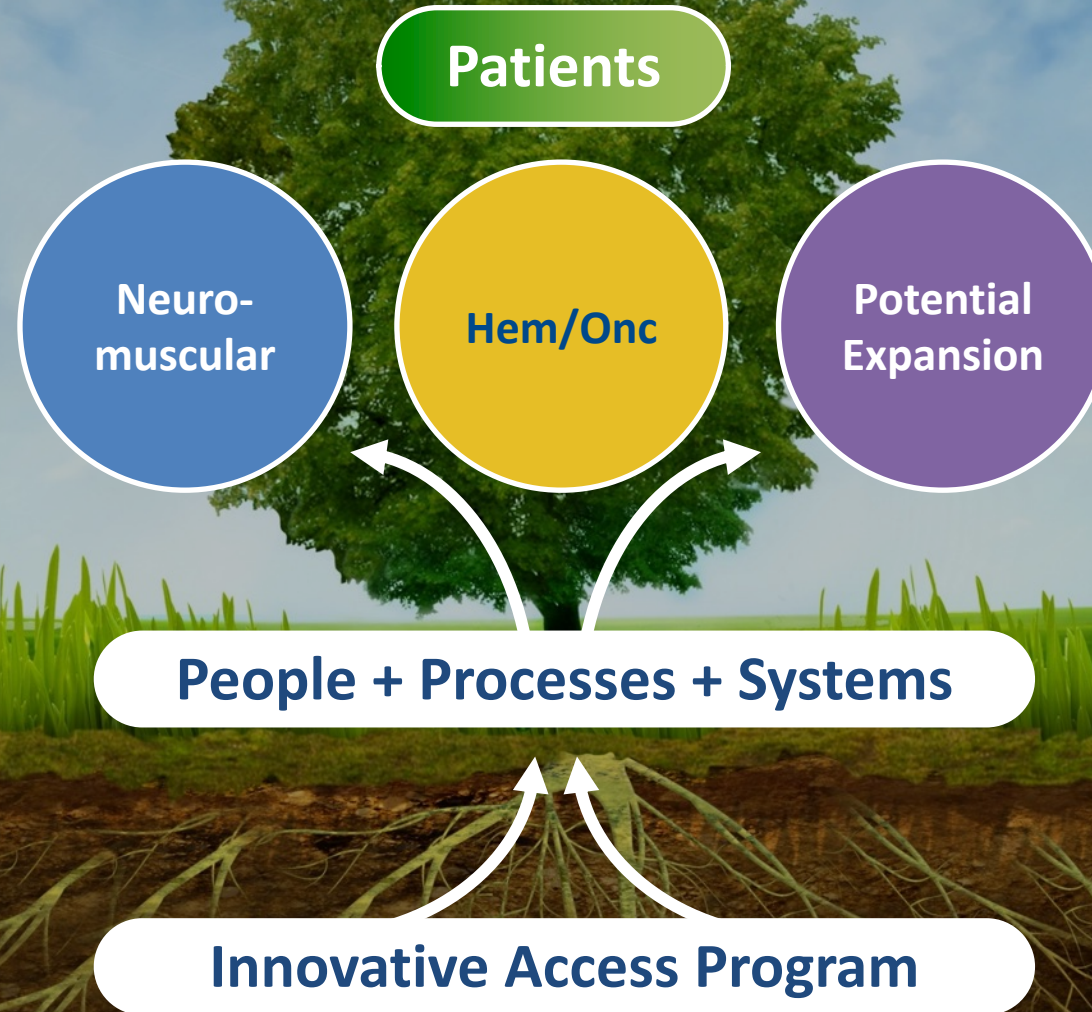
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# Agenda

- argenx 2021
- Pipeline update
- Financial results
- Outlook 2019-2020
- Q&A



# Deep Proprietary Pipeline of Highly Differentiated Product Candidates

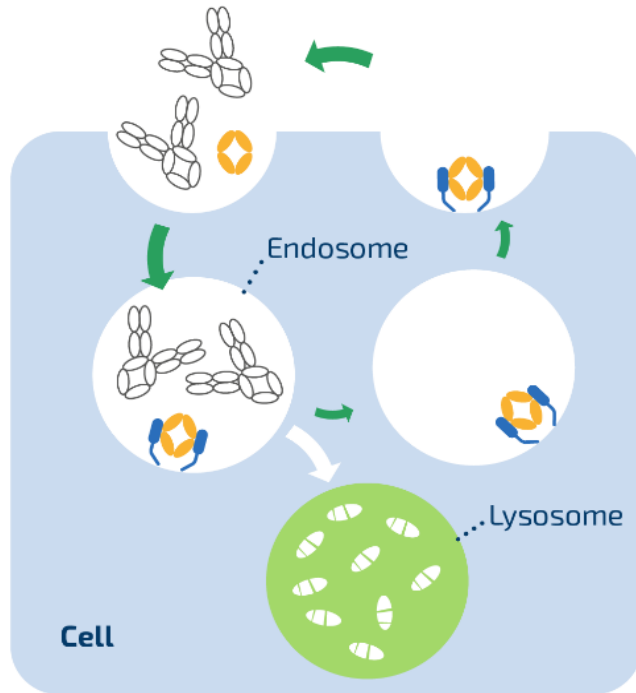
Targeting high-value rapid-growth markets



Product Candidate	Target	Indication	Preclinical	Phase 1	Phase 2	Phase 3	BLA	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 and registration-directed trial start 2H19
ARGX-117	C2	Severe Autoimmunity IV/ENHANZE® SC						CTA filing YE19
ARGX-118	Galectin-10	Airway Inflammation						Lead selected

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

Human IgG1 Fc fragment with proprietary ABDEG™ mutations



Antibody



efgartigimod



FcRn



## 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 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



## Primary objective

MG Activities of Daily Living (MG-ADL) Score

## Secondary objectives

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

**Neurology**<sup>®</sup> 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 Immune Thrombocytopenia – Phase 3 ADVANCE Trial To Start



First of two registration Phase 3 trials – start in 2H19

- Randomized, double-blind, placebo-controlled, multicenter trial enrolling up to 158 adult patients with primary ITP
- Enrolling patients with platelet levels  $<30 \times 10^9/L$  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



## Primary objective

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

## 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: Global Collaboration With Halozyme

ENHANZE<sup>®</sup> drug delivery technology – exclusive rights to products targeting FcRn



Phase 1 healthy volunteer study started – data expected year-end 2019

- Administration of ENHANZE<sup>®</sup> SC formulation of efgartigimod
- To evaluate safety, pharmacokinetics, pharmacodynamics and bioavailability
- Potential next steps:
  - Discuss bridging strategy for IV formulation with authorities
  - Two SC formulations in a patient setting: maintenance product and standalone SC product

# Efgartigimod In Pemphigus Vulgaris – Phase 2 Ongoing

Cohort 3 enrolling – 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:**

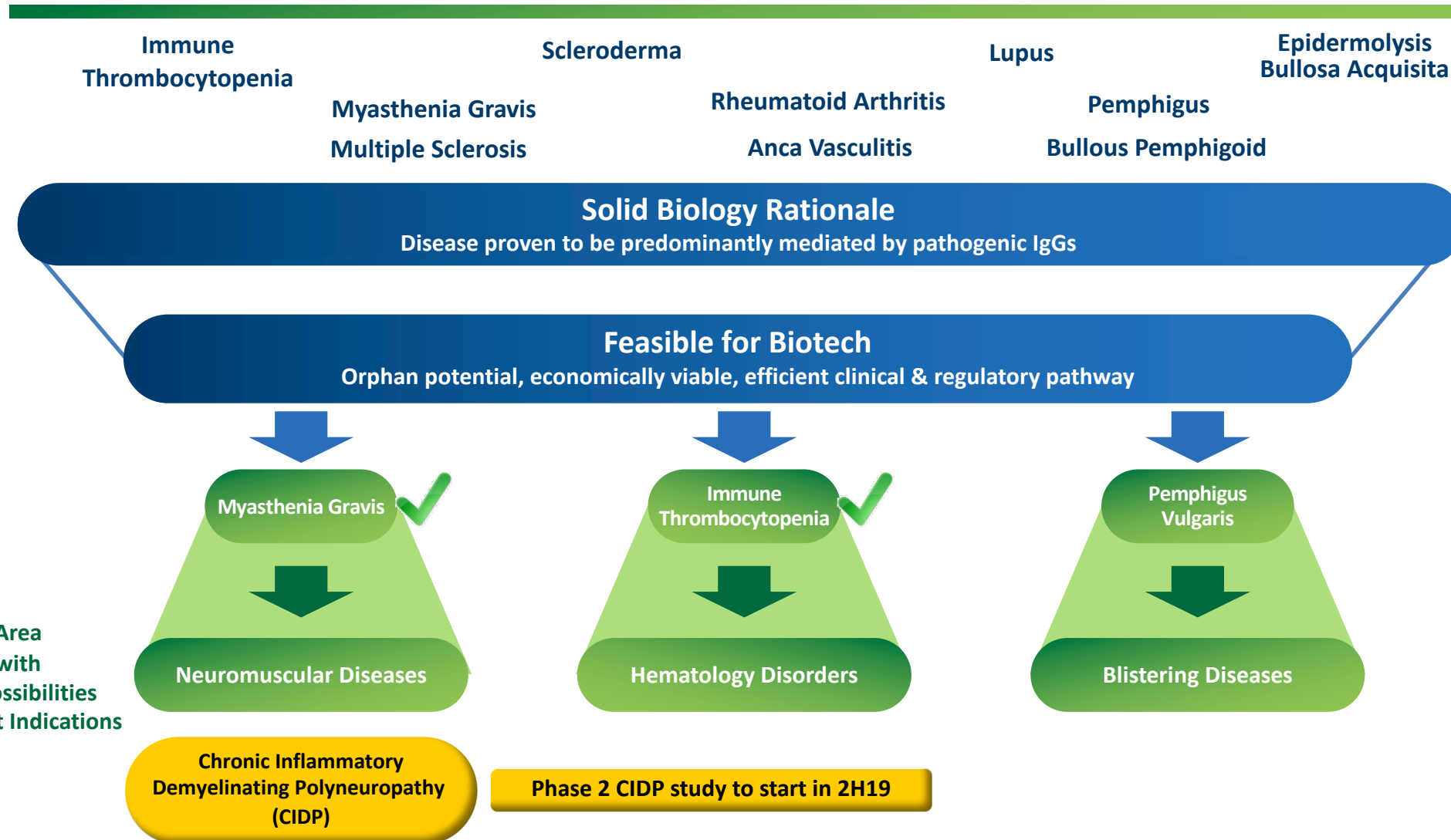
- Mean max reduction in Pemphigus Disease Area Index (PDAI) score: 55%
- Mean max decrease in pathogenic IgGs: 57%

## **Favorable tolerability profile**

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

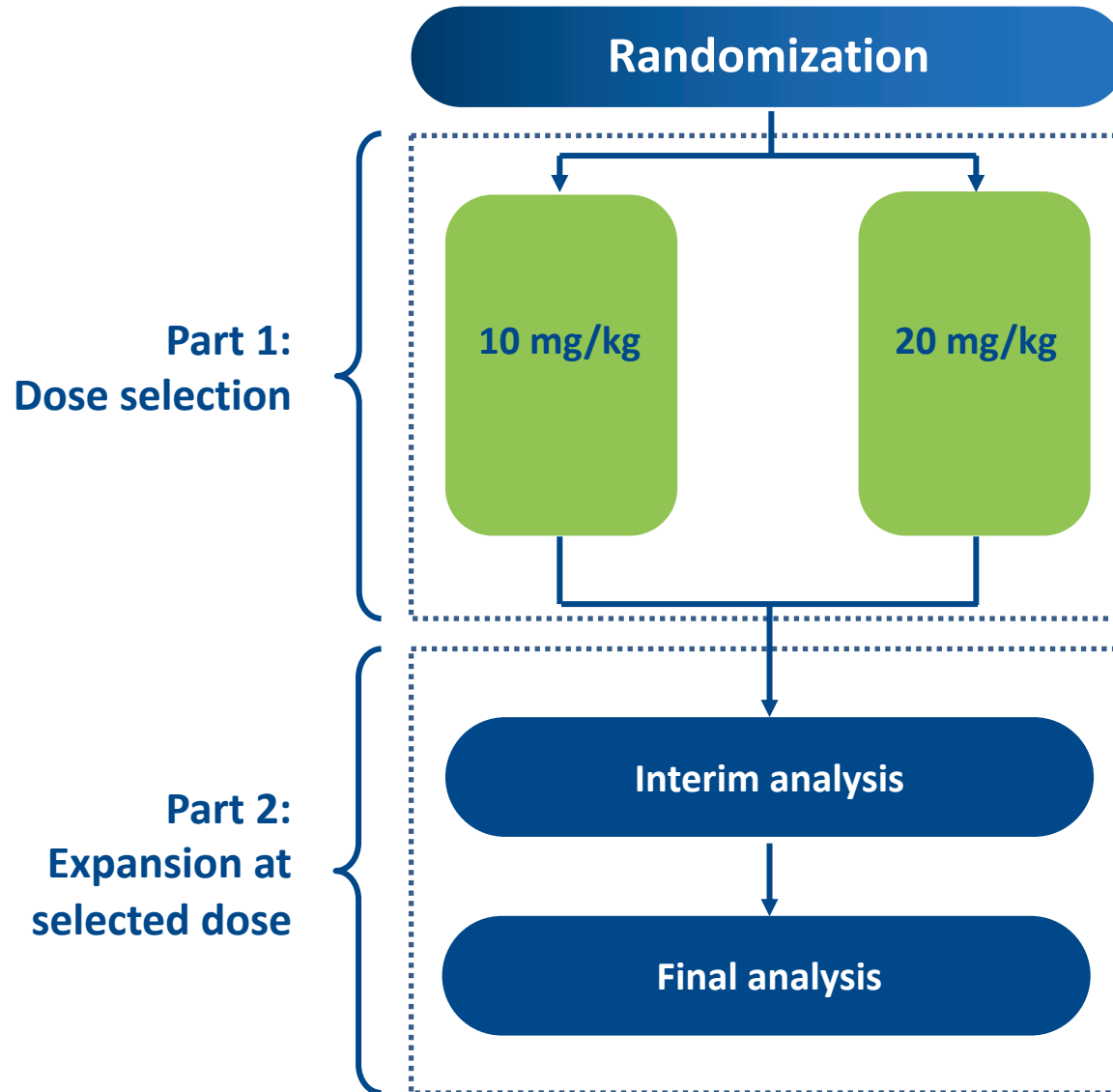
# Efgartigimod: a Pipeline-In-a-Product Opportunity

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



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

Phase 2 and registration-directed trial in acute myeloid leukemia (AML)



**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 in US:** second half 2019

# ARGX-117 – C2 Inhibitor With Pipeline-In-a-Product Opportunity

CTA filing end 2019 – first subject expected to be dosed 1Q20

1

## Novel Target Biology

Complement component C2  
UMC Utrecht: Erik Hack

5

## Franchise Structure

Well-positioned within  
argenx franchises

First indication: Multifocal  
Motor Neuropathy (MMN)

4

## Maximum Value per Asset

Severe autoimmune diseases

2

## Integrated Antibody Discovery Suite

Sweeping antibody

3

## Rapid Pipeline Expansion

Exercised option to bring  
ARGX-117 in house

Halozyme  
Option exercised for C2  
ENHANZE® SC technology



# ARGX-118 – Immunology Breakthrough In Airway Inflammation

Lead optimization – peer reviewed publications

1

## Novel Target Biology

Galectin-10  
VIB: Bart Lambrecht

Science nature The NEW ENGLAND JOURNAL of MEDICINE

5

## Preclinical Development

Lead optimization



2

## Integrated Antibody Discovery Suite

Charcot-Leyden Crystal dissolving  
SIMPLE Antibody™

3

## Rapid Pipeline Expansion

Exercised option to bring  
ARGX-118 in house

4

## Maximum Value per Asset

Range of immunology indications,  
including severe asthma

## Accessing Novel Targets Through Collaboration

argenx

Top Academic Institutions & Biotechs



### Antibody Expertise

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

### 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

ARGX-112



ARGX-116

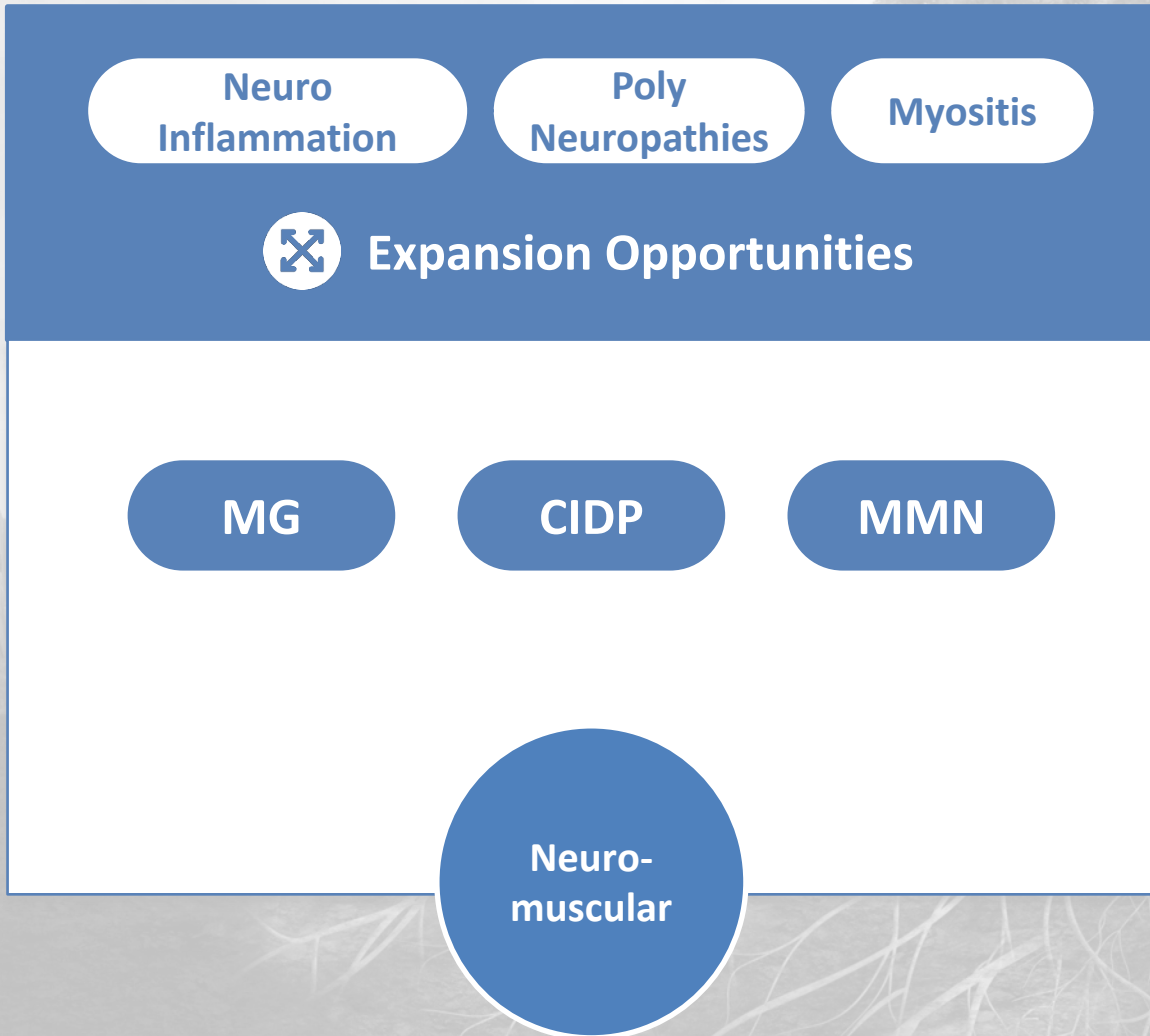
ARGX-114

STATEN



5-10 ongoing programs at any given time

# Building Immunology Franchises



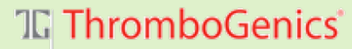


# Building The Experienced, Diverse Organization

Business Analytics



Distribution



Finance



Patient Advocacy



Human Resources



Legal / Compliance



Marketing



Market Access



Pharmacovigilance



Strategic Insight



Sales Leadership



Regulatory Affairs



Medical Affairs



Japan GM



EU Commercial Dev Leader



# Half Year 2019 Financial Results

<i>in thousands of €</i>	Six months ended June 30		Variance
	2019	2018	
Revenue	€ 43,532	€ 17,910	€ 25,622
Other operating income	7,767	2,588	5,179
<b>Total operating income</b>	<b>€ 51,299</b>	<b>€ 20,498</b>	<b>€ 30,801</b>
Research and development expenses	(78,304)	(34,371)	(43,933)
Selling, general and administrative expenses	(27,462)	(11,514)	(15,948)
<b>Operating loss</b>	<b>€ (54,467)</b>	<b>€ (25,387)</b>	<b>€ (29,080)</b>
Financial income	7,210	1,256	5,954
Exchange gains	2,486	4,024	(1,538)
<b>Loss before taxes</b>	<b>€ (44,771)</b>	<b>€ (20,107)</b>	<b>€ (24,664)</b>
Income tax (expense)/benefit	(350)	31	(381)
<b>Loss for the period and total comprehensive loss</b>	<b>€ (45,121)</b>	<b>€ (20,076)</b>	<b>€ (25,045)</b>
Net increase/(decrease) in cash, cash equivalents and current financial assets compared to year-end 2018 and 2017	€ 379,714	€ (20,922)	
<b>Cash, cash equivalents, current financial assets at end of the period</b>	<b>€ 944,283</b>	<b>€ 338,852</b>	

# Multiple Value-Creating Milestones Through 2020

2H19

Data ENHANZE® HV

Start 1<sup>st</sup> of 2 Ph 3 ITP

Start Ph 2 CIDP & KOL event

Start Ph 2 AML

ARGX-117 CTA Filing

2020

Data Ph 2 PV

Data Ph 3 ADAPT gMG

5<sup>th</sup> Indication

Development Update

ARGX-119

- Efgartigimod
- Cusatuzumab
- New Assets

\$1.05B in Cash – Funded Into 2021  
Building a Fully Integrated Biopharma

# Q&A

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