

FY2019 Financial Results & 4Q19 Business Update February 27, 2020

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# Agenda

- argenx 2021
- Pipeline update
- Commercial launch preparation for efgartigimod in gMG
- Financial results
- Q&A

## argenx Today: Late-Stage Biotech Building Towards Commercial Success



argenx 2021: Reaching Patients

**Late-Stage Pipeline** 

Therapeutic franchises

**Global expansion** 

Fast Track
Designation

FcRn leadership

MG

ITP

CIDP

PV

- ADAPT fully enrolled; data expected mid-2020
- 3/3 beachhead indications
- MyRealWorld<sup>TM</sup> MG study

**Cusatuzumab strategic alliance** 

**Immunology Breakthroughs** 

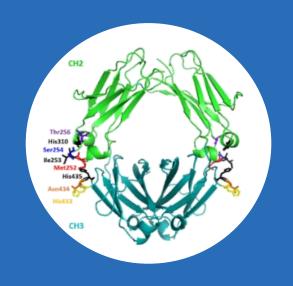
**Well-Capitalized** 

Two new pipeline assets from IAP

Raised over \$550M – Cash: €1.3B

## **Building Differentiation Every Step Of The Way**









Molecule Design:
Innovative Access Program

Clinical Development: Thoughtful ADAPT Design

**Commercial Approach:**Real-world Evidence Study

## **Efgartigimod: Unique Molecule Design Leads To Differentiated Profile in Phase 2**

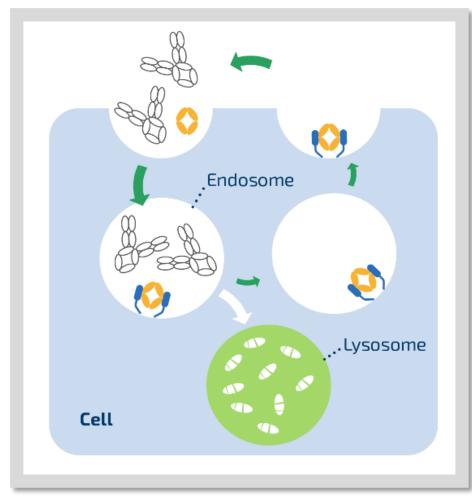


# **Efficacy**

3/3 beachhead indications

# **Safety**

No class effect



# Convenience

Potential optionality for patients





efgartigimod



## **Cusatuzumab Strategic Alliance With Janssen**



**Blocking CD70-CD27 signalling Blocking release of soluble CD27** NK effector cell Cusatuzumab Leukemia **Killing cells** cell **CD70** Leukemia cell

Joint development plan focused on AML, MDS and other heme malignancies

Upfront \$300M + \$200M equity @ 20% premium, up to \$1.3B in milestones, double digit royalties OUS

50% of US economics on a royalty basis, up to 50% commercial efforts

- First two trials underway on time and as planned
- Additional trials to start in 2020 in AML settings and subpopulations, and MDS

Achieved first milestone payment under collaboration for enrollment progress in CULMINATE

## **Innovative Access Program: Our Strategy To Grow Our Pipeline**



#### **Accessing First-in-Class Targets by Collaborating with Leading Research Biologists**

argenx

#### **Antibody Expertise**

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

**Academic Institutions & Biotechs** 

#### **Disease Biology Expertise**

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

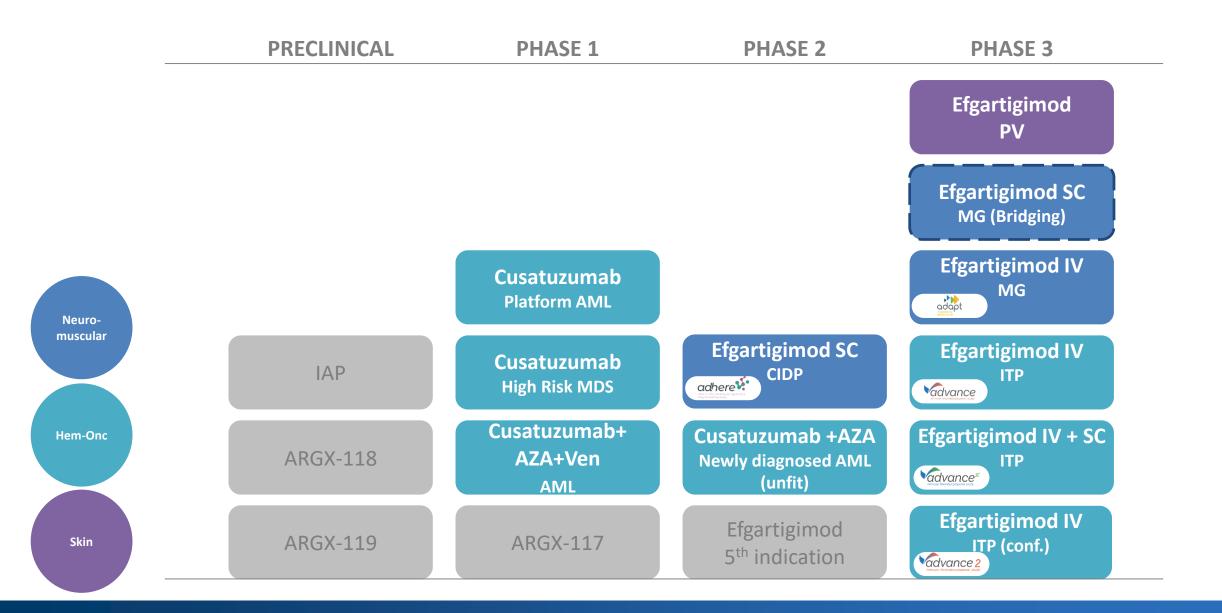
#### Co-creating immunology solutions: building beyond each individual contribution



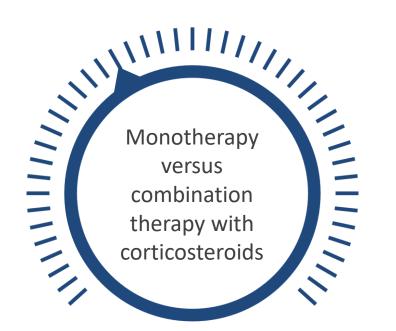
8 assets from Innovative Access Program have delivered value to argenx

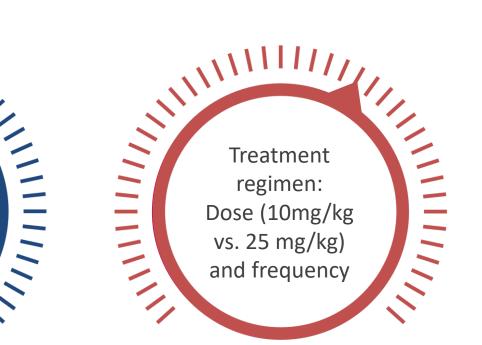
## **2020 View Of Pipeline: Poised To Have Five Phase 3 Trials Underway**

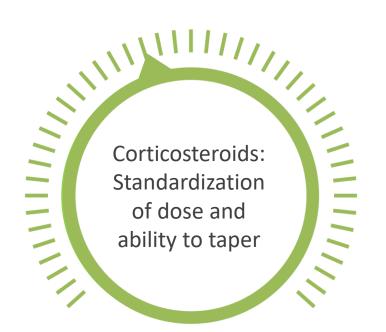












# **Efgartigimod Synergizes With Low-Dose Steroids In Pemphigus in Phase 2**

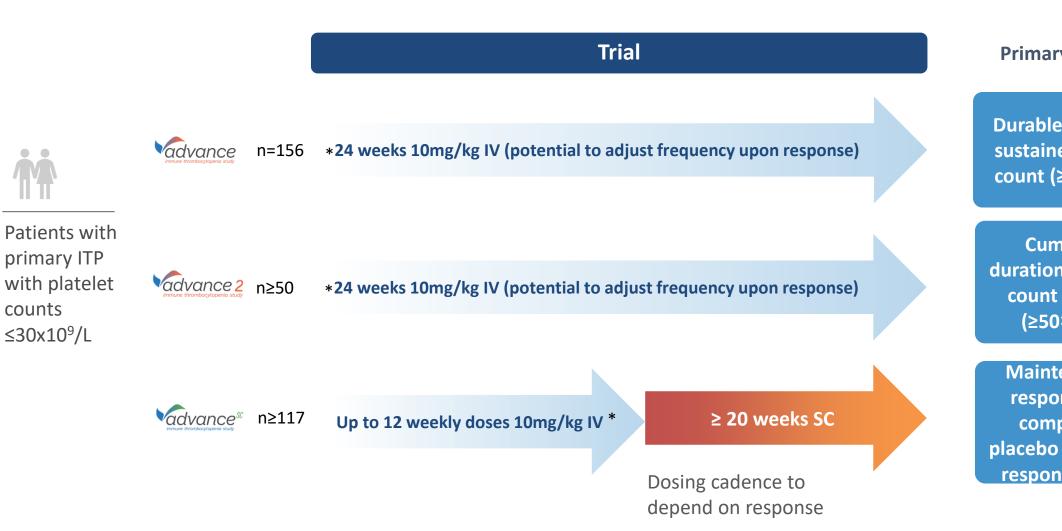


Fast onset of action	78% disease control (18/23 patients) – majority after 1-2 infusions Median time to DC: 14 to 15 days (mono/combo therapy)				
Deep responses	70% complete clinical remission (5/7 patients) on optimized dosing * Time to CR: 2-10 weeks				
	Mean maximum PDAI improvement in responders >60% to >85% (mono/combo therapy)				
	Strong steroid sparing potential demonstrated				
Favorable tolerability	Determined by independent monitoring committee				
Potential synergy	Efgartigimod clears a-Dsg antibodies/Steroids stimulate Dsg synthesis				

<sup>11</sup> 

## **ITP Phase 3 ADVANCE: Evaluating IV + SC Maintenance Dosing**





**Primary objective** 

Durable response: sustained platelet count (≥50×10<sup>9</sup>/L)

Cumulative duration of platelet count response (≥50×109/L)

Maintenance of response of SC compared to placebo after initial response with IV

## **CIDP Phase 2 ADHERE: Potential For Development Acceleration**





**Identify patients with active CIDP** 

Confirm IgG autoantibody involvement

Document efficacy & safety efgartigimod vs placebo

#### **Treatment period**

**Open-label** 

Stage A

Placebo-controlled

Stage B (stage A responders only)



Placebo weekly SC



**Efgartigimod weekly SC** 

Up to 48 weeks

#### Screening

Confirmation
 of diagnosis by
 independent
 committee

#### **Run-in period**

- Worsening of disease within 12 weeks after drug withdrawal (INCAT, I-RODS, grip strength)
- Newly diagnosed/treatment naïve skip run-in period

≤4weeks ≤13weeks

Up to 12 weeks, until clinical improvement (ECI)

**Efgartigimod weekly SC** 

Efficacy analysis based on relapse (adjusted INCAT)

Study endpoint with 88 relapse events in stage B

N=sample size estimation ~120-130

Followed by Open Label Extension study

## **ADAPT Trial: Built For Patients Based On Strengths Of Efgartigimod**



We listened to stakeholders...



Request to be tailored, convenient, cost-effective

...and built on observed attributes of efgartigimod

#### Phase 2 MG data:

#### Fast onset of action

Responded within first four weeks

Clinical response in 83% of patients

#### **Durable response in 75% of patients**

Sustained for at least 6 weeks

**Promising tolerability** 

## **Innovative ADAPT Design: Clinical Trial Designed To Meet Clinical Practice**





**Patient** population consistent with Phase 2

gMG patients (MG-ADL≥5)

**Stratified for** AChR+ or AChRand background therapy (n=167 total)

**Enrollment** Completed

Primary endpoint readout at week 8 Duration of benefit measured over 26 weeks

**Treatment Cycle** efgartigimod 8 weeks

26 weeks

**Individualized treatment cycles** 

Time between cycles determined by duration of sustained treatment benefit

**Open-label Extension** Retreat as needed to simulate clinical practice

10mg/kg IV efgartigimod



Primary endpoint (AChR+): % responders after first treatment cycle

10mg/kg IV

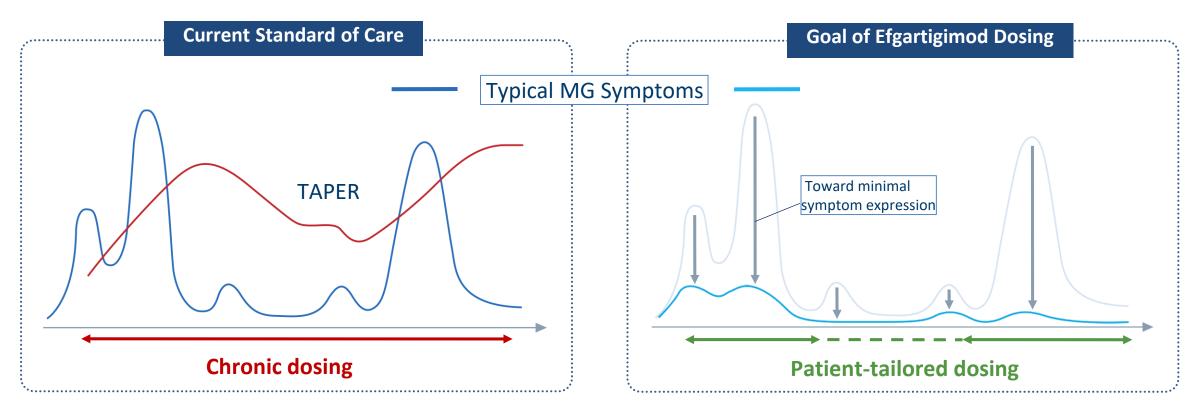
or

placebo

Responder: ≥2 ADL points for at least 4 consecutive weeks any time within initial treatment cycle

## **Efgartigimod Has Potential To Offer Tailored Treatment Approach In MG**





- Fast-acting steroids and slow-acting immunosuppressants
- Balancing symptom suppression and side effects

- Tailored regimen matches variability of MG
- Time between cycles is individualized
- Period of sustained therapeutic benefit between cycles can offer flexibility



#### First of its kind in MG



Global prospective – longitudinal - observational



Voice of ≥2000 patients - digitally



Patient perspective on diagnosis, treatment, symptom, economic and humanistic burden

## The Right Team In Place To Launch Efgartigimod



COO leading commercial organization

Commercial leaders hired across all key functions

Field-based medical research liaisons in place

**Stepwise salesforce ramp-up** 

#### Significant product launch experience



































































#### **Preparing for Global Launch**



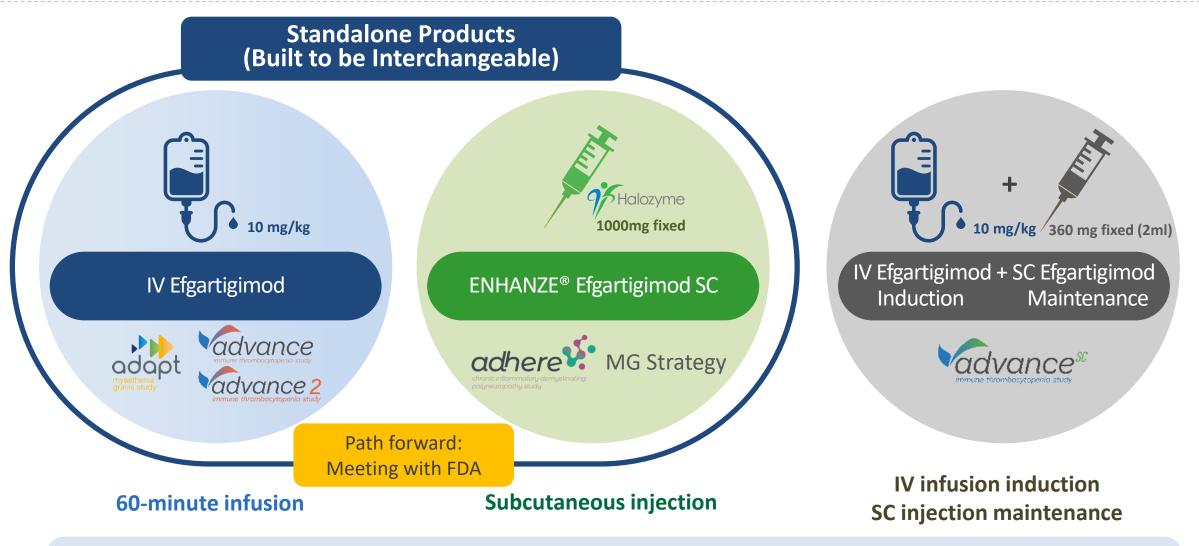




## **Efgartigimod Portfolio: Multiple Formulations In Development**







Three Formulations Available for Use in Future Studies

# **Y2019 Financial Results**



		Year Ended December 31,				
in thousands of €		2019	2019 2018		Variance	
Revenue	€	69,783	€	21,482	€	48,301
Other operating income		12,801		7,749		5,052
Total operating income		82,584		29,231		53,353
Research and development expenses		(197,665)		(83,609)		(114,056)
Selling, general and administrative expenses		(64,569)		(27,471)		(37,098)
Fair value gains on financial assets at fair value through profit or loss		1,096		_		1,096
Operating loss	€	(178,554)	€	(81,849)	€	(96,705)
Financial income		14,399		3,694		10,705
Financial expense		(124)		_		(124)
Exchange gain/(losses)		6,066		12,308		(6,242)
Loss before taxes	€	(158,213)	€	(65,847)	€	(92,366)
Income tax expense	€	(4,752)	€	(794)	€	(3,958)
Loss for the year and total comprehensive loss	€	(162,965)	€	(66,641)	€	(96,324)
Net increase in cash, cash equivalents and current financial assets compared to						
year-end 2018 and 2017		771,252		204,795		
Cash, cash equivalents and current financial assets at the end of the period		1,335,821		564,569		



1

ADAPT PH3 MG CLINICAL DATA - PREPARE FOR LAUNCH

2

**EXECUTE PIPELINE: 5 REGISTRATIONAL AND 7 PHASE 1-2 TRIALS** 

3

**EXPAND THROUGH INNOVATIVE ACCESS PROGRAM** 



Q&A