Developing Highly Differentiated Antibody Therapeutics

argenx corporate presentation

Tim Van Hauwermeiren, CEO

JMP Life Sciences Conference, New York
June 20, 2018

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 and clinical trials and the status and related results thereto, future results of operations and financial positions, 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 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 or

Company Highlights

Differentiated therapeutic antibodies pioneering in severe autoimmune diseases & cancer

Novel concept in autoimmunity

- **ARGX-113**: first-in-class FcRn antagonist targeting array of IgG mediated AI diseases
  - Phase 1: favorable tolerability profile; IgG reduction up to 85%
  - Phase 2: achieved proof of concept in myasthenia gravis, ongoing in immune thrombocytopenia and pemphigus vulgaris

Deep pipeline with multiple shots on goal

- **ARGX-110:** first-in-class CD70 antagonist in Phase 1/2 in CTCL and AML
- 4 clinical stage programs; 3 preclinical programs; Innovative Access Program

Powerful technology suite

- **SIMPLE Antibody™:** Human V-regions sourced from llama unlock **novel & complex targets**
- **NHance®, ABDEG™, POTELLIGENT®:** Fc engineering to augment natural properties of antibodies

Validating selective partnerships

- abbvie: **ARGX-115** (Immuno-oncology-focused novel target GARP)
  - $40mm upfront and up to $625mm in potential milestone payments
- Additional partnerships designed to maximize value of platform in non-core areas

Well financed to execute plan

- **Strong cash position:** €347 mm March 31, 2018
- Blue chip investor base: more than 60% U.S. Shareholders
- 32.40 mm shares outstanding

Upcoming milestones

- **ARGX-113**
  - Ph2 MG: End of Ph2 meeting with FDA, start Ph3 before year end
  - Ph1 subQ HV study: feasibility of IV loading dose followed by subQ maintenance dose
  - Ph2 PV: interim data 2H18
  - Ph2 ITP: top line data 2H18
    - Amendment 1: follow up period extended from 8 wks to 21 wks
    - Amendment 2: patients can roll over in open label (re)treatment arm of 1 year

- **ARGX-110**
  - Ph2 newly diagnosed, elderly AML patients, unfit for chemotherapy in combo with Vidaza
    - Selected dose: 10 mg/kg, recrtuitment of an initial 21 patients

- Other **pipeline progress**

Generating Differentiated Antibody Candidates

Disciplined business model in severe auto-immune and cancer area

NOVEL/COMPLEX TARGETS

FcRn • CD70 • c-MET • IL-22R • GARP • ApoC3

TOP NOTCH COLLABORATORS

Renowned academics • Innovative biotechs

Innovative Access Program

DIFFERENTIATED ANTIBODIES + **EFFICIENT DEVELOPMENT PATH**

ADVANCED TECHNOLOGY SUITE

SIMPLE Antibody™ • NHance® • ABDEG™ • POTELLIGENT®

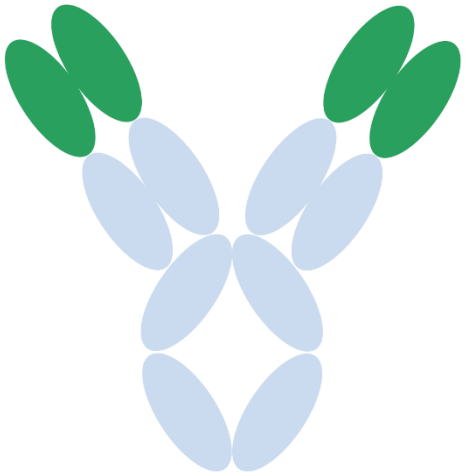
WINNING (ORPHAN) INDICATIONS

Strong biology rationale • Clear endpoints

MG • ITP • PV • AML • CTCL

Augmenting Intrinsic Therapeutic Properties Of Antibodies

Antibody	Technology Role	Suite of technologies
V-region	Unlock novel and complex targets	**SIMPLE Antibody™ Platform** • Llama immune system delivers V-regions with high human homology • Highly diverse antibody output covers a multitude of target epitopes
Fc region	Modulate immune response	**NHance®** • Extends half-life / PD effect • Enhances tissue penetration **ABDEG™** • Clears disease target • Clears autoantibodies **POTELLIGENT®** • Boosts cell killing



We apply our unique suite of technologies to create differentiated product candidates against novel targets

Klarenbeek et al. 2015, mAbs
Basilico et al. 2014, J Clin Inv.

Deep Pipeline In Severe Autoimmune Diseases and Cancer

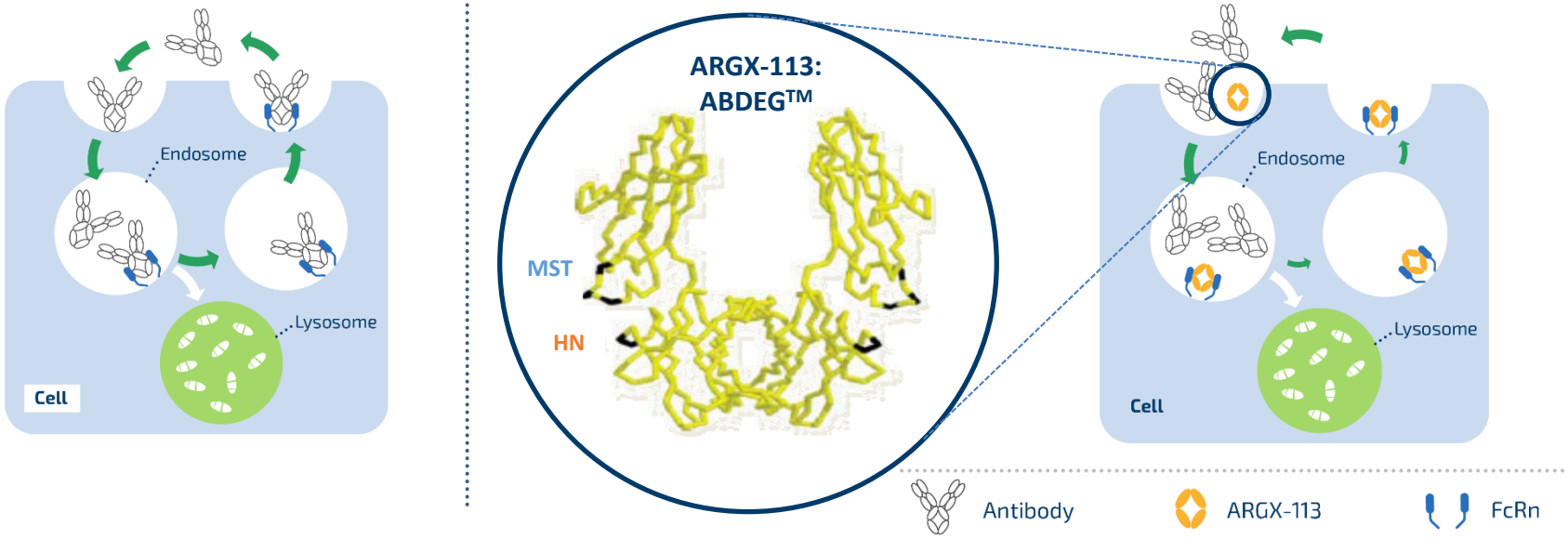
Product Candidate	Target	Indication	Preclinical	Phase 1	Phase 2	Phase 3	Next Milestone / Commentary
Wholly-Owned Product Candidates							
ARGX-113 (efgartigimod)	FcRn	Myasthenia Gravis					Positive Phase 2 results ✓
		Immune Thrombocytopenia					2H18: Phase 2 topline results
		Pemphigus Vulgaris					2H18: Phase 2 interim data
		Chronic Autoimmune Diseases			SubQ Formulation		2H18: Phase 1 interim data
ARGX-110 (cusatuzumab)	CD70	T-Cell Lymphoma			Phase 1/2		2H18: Phase 2 topline results CTCL
		Acute Myeloid Leukemia			Phase 1/2		2H18: Transition into Phase 2 in AML/MDS
ARGX-117	Novel complement target	Severe Autoimmune Diseases					Antibody-mediated autoimmune diseases Complementary to ARGX-113
ARGX-111	c-MET	Solid Tumors / Blood Cancer					Available for partnering
Partnered Product Candidates							
ARGX-109 (gerilimzumab)	IL-6	Rheumatoid Arthritis					Development for Chinese market
ARGX-112	IL-22R	Skin Inflammation					Eligible for up to ~€100mm in milestones and tiered royalties
ARGX-115	GARP	Cancer Immunotherapy					Received $50mm so far; eligible for up to $625mm milestones & tiered royalties
ARGX-116	ApoC3	Dyslipidemia					Eligible for double-digit royalties and exclusive option to license the program

- **Innovative Access Program**: 6 live programs
- We have an antibody discovery alliance with Shire focused on multiple rare disease targets

ARGX-113: A Pipeline-in-a-Product Opportunity

ARGX-113 Exploits The Natural Fc/FcRn Interaction Site, Leveraging Our Proprietary ABDEG™ Technology

IgG antibodies recycle through FcRn(1)… **…ARGX-113 potently blocks FcRn…** **…leading to IgG elimination**



- ARGX-113 is a human IgG1 Fc-fragment that utilizes ABDEG™ Fc engineering technology(2)(3)
- ARGX-113 cannot engage Fcγ receptors when bound to its target FcRn
- ARGX-113 targets and binds to FcRn blocking the recycling of IgG leading to an elimination of IgG antibodies
- Pathogenic IgG antibodies mediate multiple autoimmune diseases

(1) Roopenian et al. 2007, Nat Rev Immunol.
(2) Vaccaro et al. 2005, Nat Biotech.
(3) argenx data

ARGX-113: Pipeline-In-Product Opportunity

Prioritizing IgG autoantibody mediated diseases

Landscape of IgG severe autoimmune diseases (selection)

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

Solid Biological Rationale
Disease proven to be predominantly mediated by pathogenic IgGs

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

Proof of Concept Value
Spill-over effect into adjacent indications

Myasthenia Gravis	Immune Thrombocytopenia	Pemphigus vulgaris
Beachhead neuromuscular diseases	Beachhead heme disorders	Beachhead blistering diseases

argenx data

Myasthenia Gravis Overview

What is Myasthenia Gravis (MG)?

- Rare autoimmune disorder; 64,000[1] patients in U.S., 55,000[2] with generalized MG (gMG)
- Severe muscle weakness
- Symptoms include: drooping eyelids, double vision, difficulty to speak/swallow, generalized muscle weakness, life-threatening choking,…

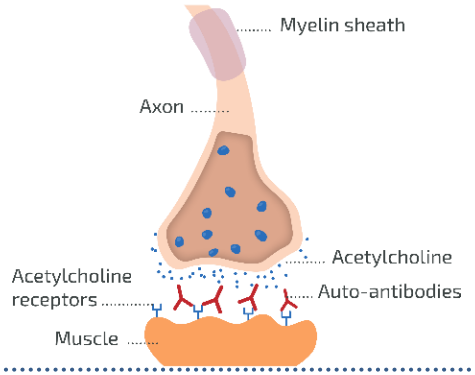
Limited current treatment options with severe side effects

- Cholinesterase inhibitors
- Corticosteroids
- Immunosuppressants
- IVIg, Plasmapheresis (exacerbations or rescue)
- Soliris®
- Thymectomy (minority of patients)

IVIg, Plasmapheresis and Soliris® place a heavy cost burden on healthcare systems (~$79,000[3] ,~$101,000[3] and ~$700,000[4])

Autoantibodies (IgG type) impact neuromuscular junctions:

- Blocking of Acetylcholine Receptors (AChRs)
- Cross-linking + internalization of AChRs
- Complement recruitment



(1) Philips et al. 2003, Ann N Y Acad Sci
(2) Drachman et al. 1993, New Eng J Med.
(3) Heatwole et al. 2011, J Clin Neuromuscul Dis.
(4) Source: Reprinted with permission by First Databank Inc. WAC = Wholesale Aquisition Cost 8/21/17

Autoantibody Levels (IgGs) Correlate With MG Disease Score

>30% autoantibody reduction clinically meaningful

Treatment*	Plasmapheresis	Immuno-adsorption	IVIg
Decrease in autoantibody levels (%) after treatment	62.6 ± 0.9	55.1 ± 3.2	28.9 ± 3.8
Decrease in disease score (%) after treatment	60.8 ± 3.5	42.4 ± 4.2	23.8 ± 3.7
Clinical efficacy rate after 14 days**	12/15	7/10	6/15
Duration of hospital stay (days)	12.80 ± 0.28	13.50 ± 0.50	16.00 ± 0.50

* Comparison between 3 cycles of Plasmapheresis/Immunoadsorption every 24h-48h and 5 cycles of IVIg every 24h
** Clinically effective if disease score has improved by >50% 14 days after treatment

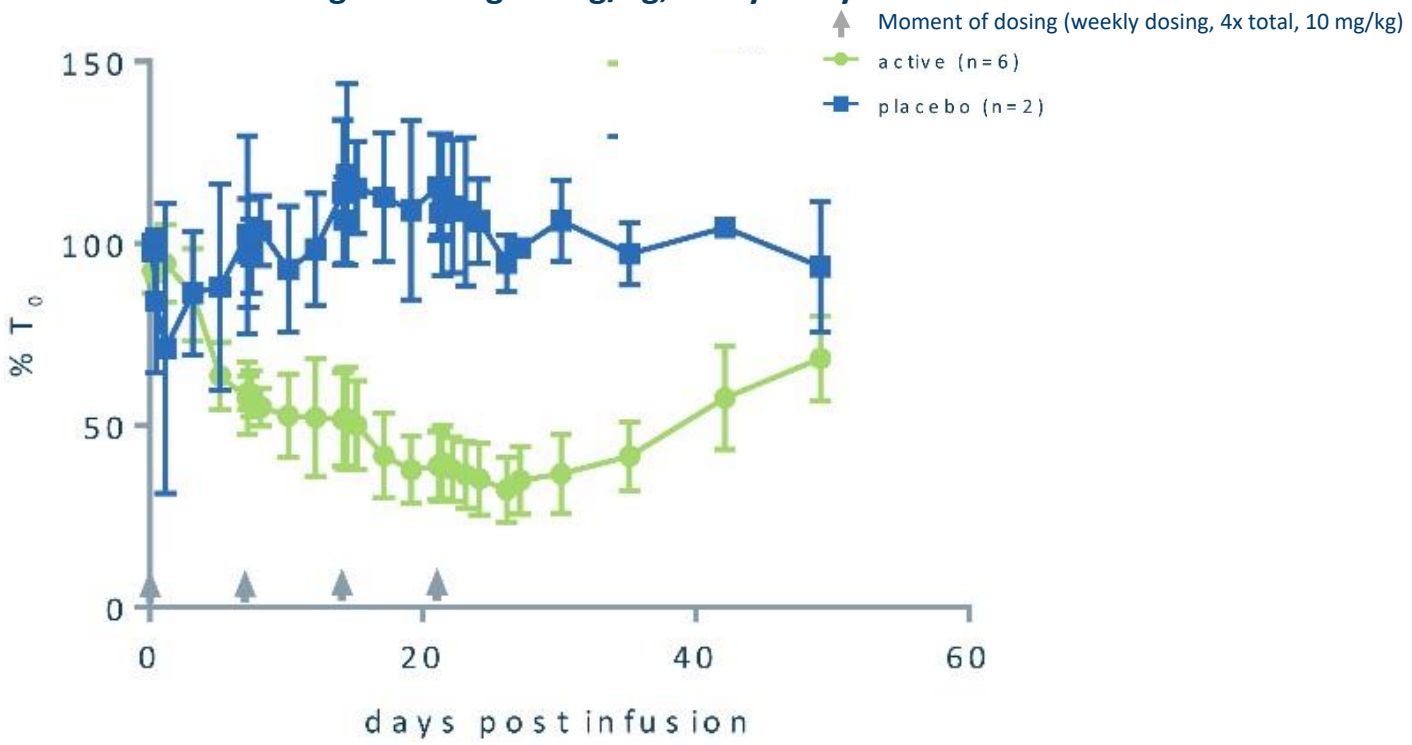
Degree of autoantibody reduction correlates with clinical improvement and reduced hospital stay

Liu et al. 2010, Ther Apher Dial.

ARGX-113: Selective and Lasting IgG Reduction

PD data multiple ascending dose (MAD) study in healthy volunteers

Total IgG - Dosing 10 mg/kg; every 7 days

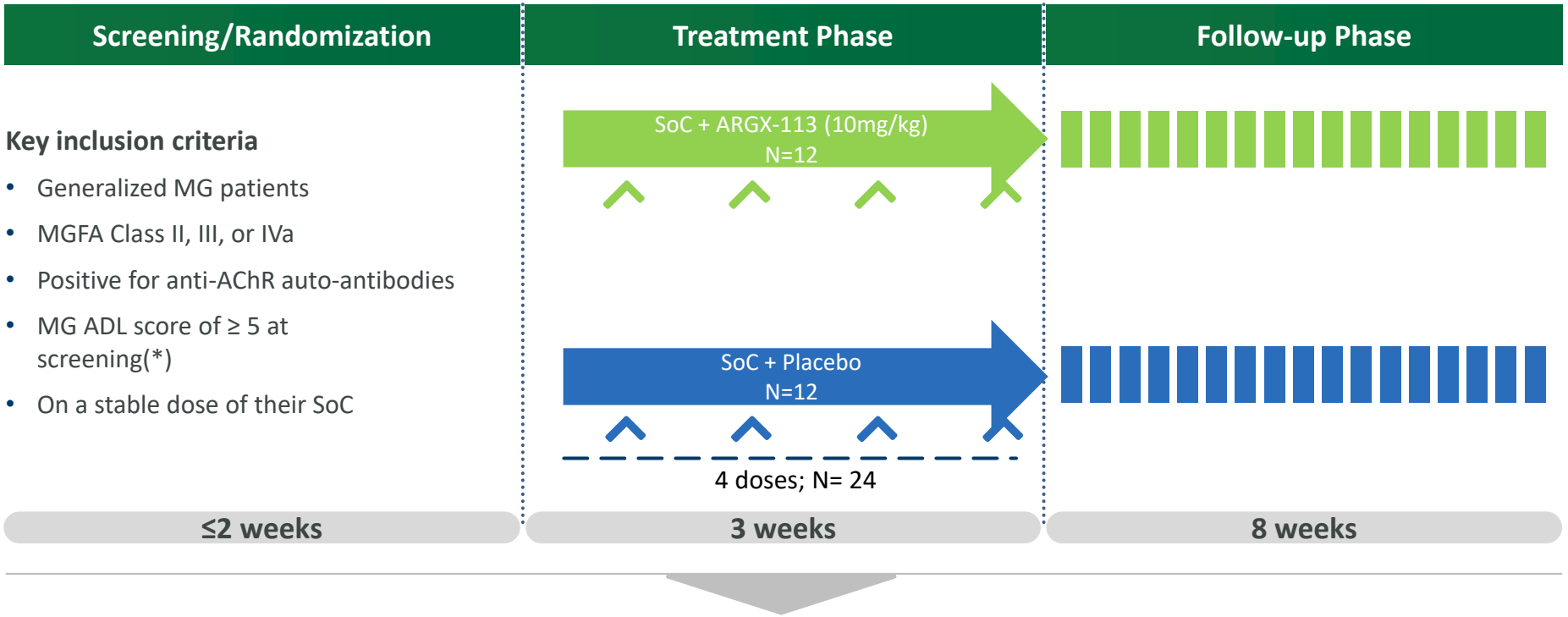


- Potent IgG reduction accross isotypes (AChR autoantibodies are IgG1/3; MuSK autoantibodies are IgG4)
- Up to 85% total IgG reduction; single dose delivers 50% total IgG reduction
- After last dose, IgG levels remain reduced by 50% or more for ~3 weeks, return to baseline after > 1 month
- Comparable data for 25 mg/kg, every 7 days (data not shown)

Myasthenia Gravis Phase 2 Trial Design

Study start-to-finish in 11 months

argenx



Primary endpoint

Secondary endpoints

Safety & tolerability	Efficacy (MG-ADL; QMG; MGC; MG-QoL)	PK	PD total IgG; pathogenic IgG	Immuno-genicity

(*) >50% of the score attributed to non-ocular items

Clinicaltrials.gov: NCT02965573, argenx data

Baseline Population and Disease Characteristics

	Placebo (N = 12)	Efgartigimod (N = 12)
Age (mean ± SD)	43.5 ± 19.3	55.3 ± 13.6
Gender (N (%))		
• Male	4 (33.3%)	5 (41.7%)
• Female	8 (66.7%)	7 (58.3%)
Race		
• Asian	-	1 (8.3%)
• Black / African American	1 (8.3%)	-
• White	11 (91.7%)	11 (91.7%)
MGFA Disease Class at Screening		
• Class II	7 (58.4%)	6 (50.0%)
• Class III	4 (33.3%)	6 (50.0%)
• Class IV	1 (8.3%)	-
Baseline QMG score (mean ± SD) (min, median, max score)	11.8 ± 5.4 (3, 12.5, 24)	14.5 ± 6.3 (6, 14, 30)
Baseline MG-ADL score (mean ± SD) (min, median, max score)	8.0 ± 2.2 (5, 8, 13)	8.0 ± 3.0 (5, 7.5, 15)
Baseline MGC score (mean ± SD)	14.5 ± 4.5	16.7 ± 8.7
Baseline MGQoL score (mean ± SD)	14.5 ± 6.1	19.7 ± 5.7
SoC		
• Acetylcholinesterase inhibitors N (%)	11 (91.7%)	12 (100.0%)
• Corticosteroids N (%)	5 (41.7%)	8 (66.7%)
• Immunosuppressants N (%)	2 (16.7%)	9 (75.0%)

Efgartigimod Safety And Tolerability Profile

2 hour infusion enabling out-patient administration

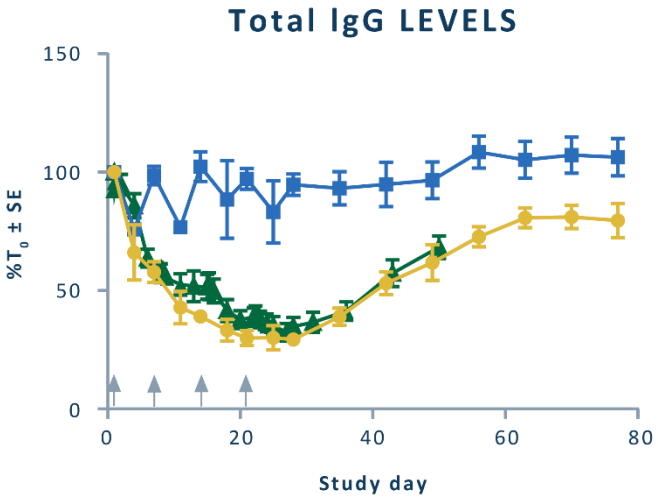
- Efgartigimod was well-tolerated in patients confirmed findings from Phase 1 healthy volunteer trial
- The TEAEs profile was balanced between efgartigimod and placebo
- TEAEs were mostly mild (grade 1) in severity; no severe AEs were reported
- No deaths, serious AEs or TEAEs leading to discontinuation of treatment were reported during the trial

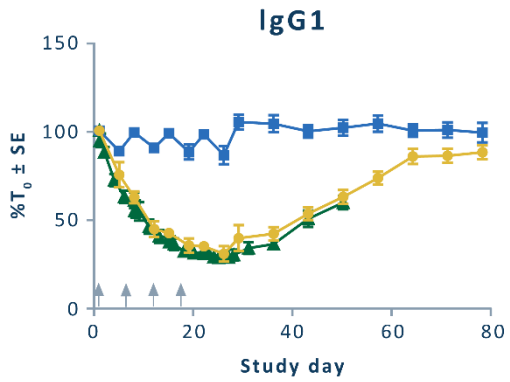
Treatment Emergent Adverse Events (TEAEs) Reported in ≥ 2 patients	Placebo (N = 12)	Efgartigimod (N = 12)
TEAEs (Total)	**10 (83.3%)**	**10 (83.3%)**
• Headache	3 (25.0%)	4 (33.3%)
• Nausea	1 (8.3%)	1 (8.3%)
• Diarrhea	1 (8.3%)	1 (8.3%)
• Abdominal pain upper	1 (8.3%)	1 (8.3%)
• Arthralgia	2 (16.7%)	-
• B-lymphocyte decrease	-	2 (16.7%)
• Lymphocyte count decrease	-	2 (16.7%)
• Monocyte count decrease	-	2 (16.7%)
• Neutrophil count increase	-	2 (16.7%)
• Myalgia	-	2 (16.7%)
• Pruritus	2 (16.7%)	1 (8.3%)
• Rhinorrhea	1 (8.3%)	1 (8.3%)
• Tooth abscess	2 (16.7%)	-
• Toothache	2 (16.7%)	-
Efgartigimod deemed related TEAEs	**3 (25.0%)**	**8 (66.7%)**
• Headache	1 (8.3%)	3 (25.0%)
• Monocyte count decrease	0 (0.0%)	2 (16.7%)
• Rhinorrhea	1 (8.3%)	1 (8.3%)

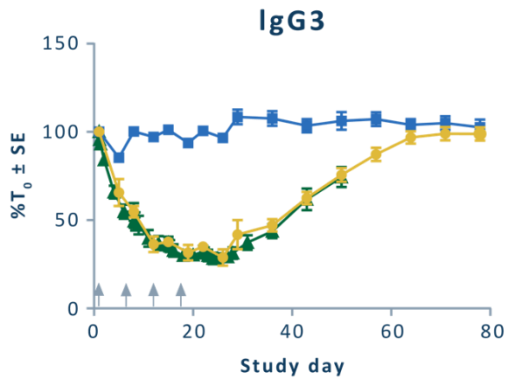
argenx data

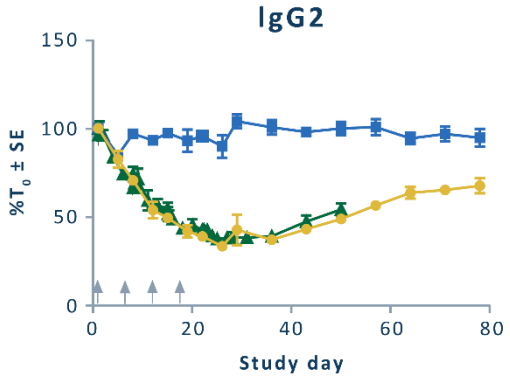
Lasting IgG Reduction

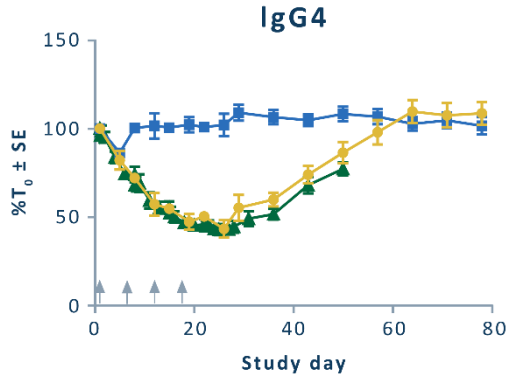
Potent IgG reduction across isotypes

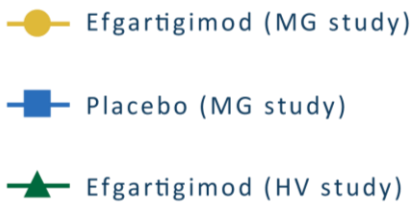










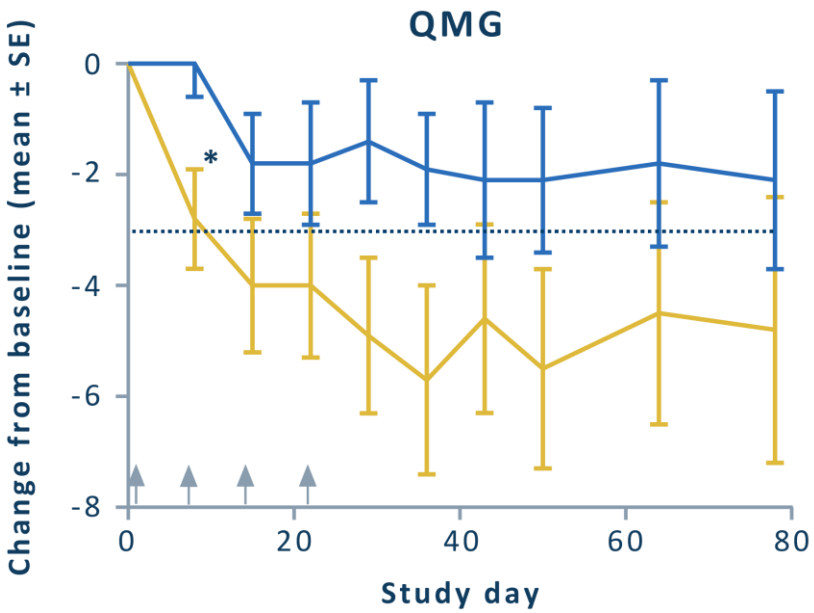


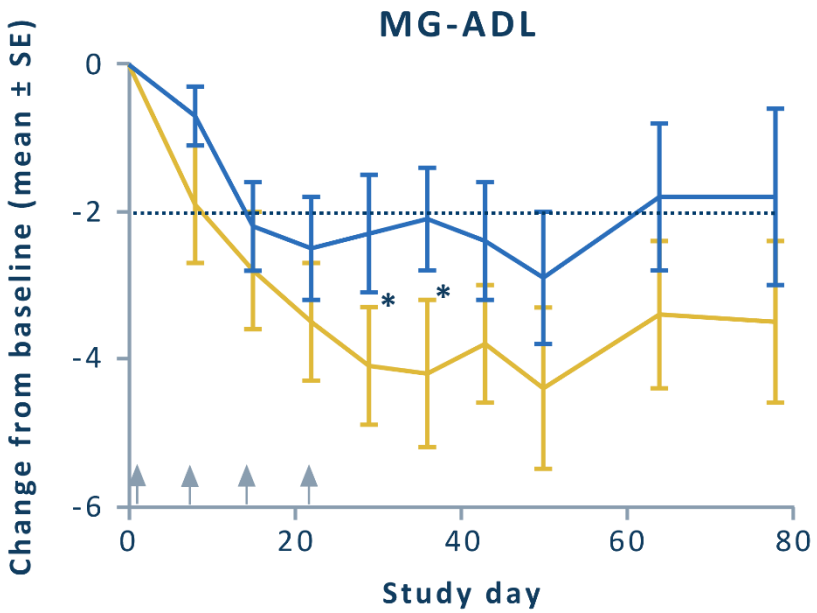
- PD effect of efgartigimod in the Phase 2 clinical trial very similar to the Phase 1 trial in healthy volunteers
- Significant IgG reduction across IgG subtypes (AChR autoantibodies are IgG1/3; MuSK autoantibodies are IgG4)
- IgM, IgA and albumin levels not affected (data not shown)

argenx data

Clinically Meaningful and Long-lasting Reduction of Efficacy Scores

QMG and MG-ADL scores





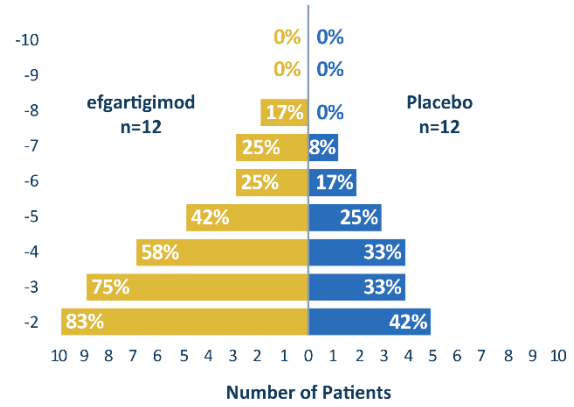
Efgartigimod Placebo * p < 0.05

- Clinically meaningful and statistically significant improvement reached in small patient population (N=24)
- Clear consistency between QMG and MG-ADL scores

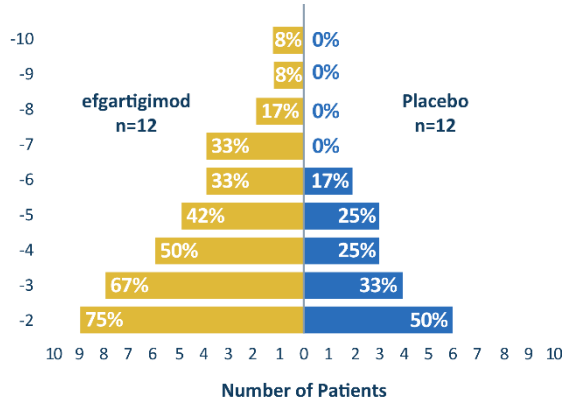
Robust Clinical Improvement Over Placebo Group

MG-ADL change from baseline

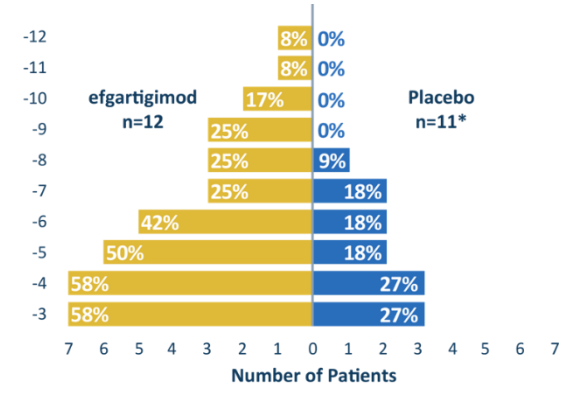
Day 29, 1 week after last dosing

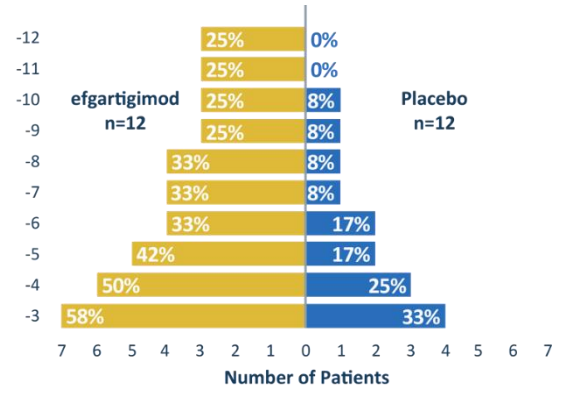


Day 36, 2 weeks after last dosing



QMG change from baseline



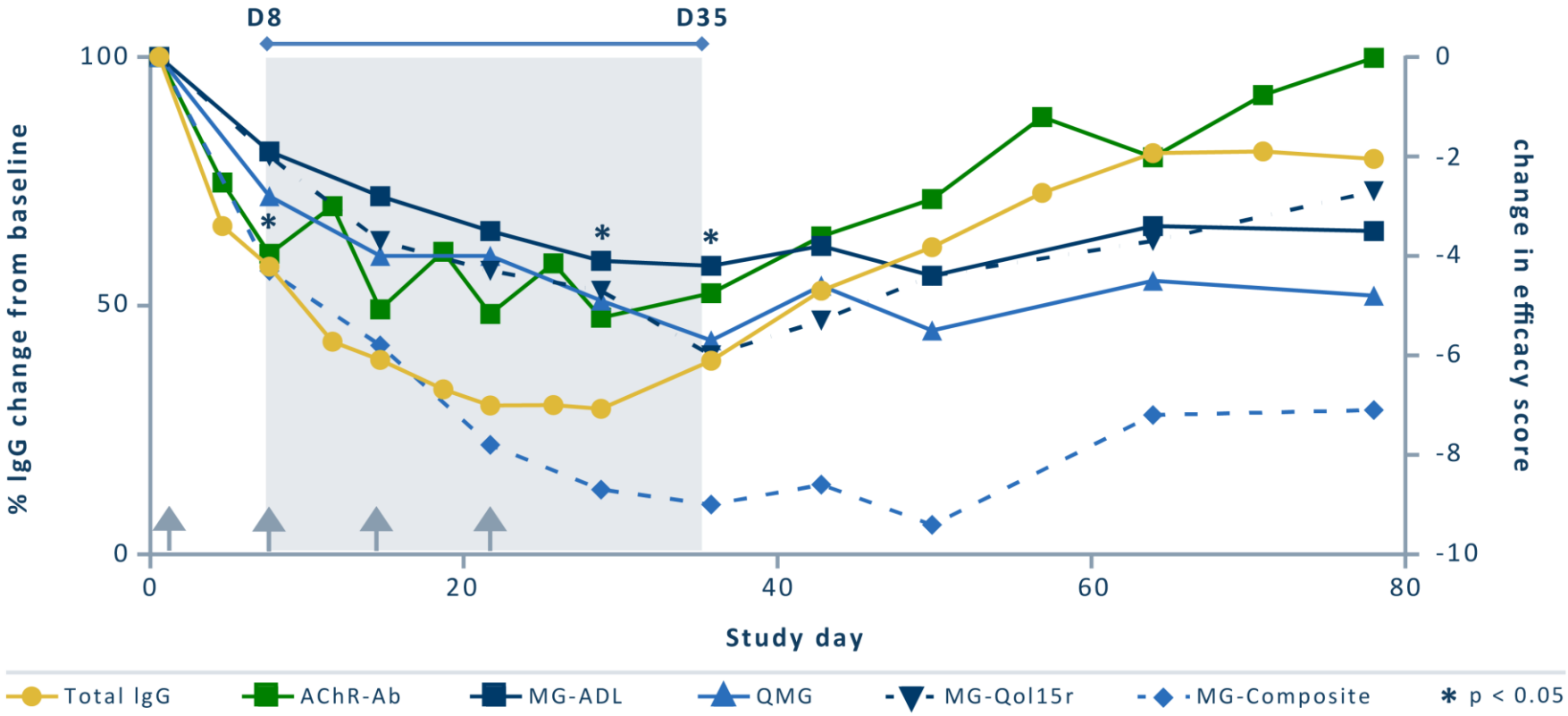


- Efgartigimod *vs.* placebo: increasing differentiation observed with increasing MG-ADL/QMG thresholds

* Missing data point of 1 patient

Total & Pathogenic IgG Reduction Correlates with Clinical Improvements

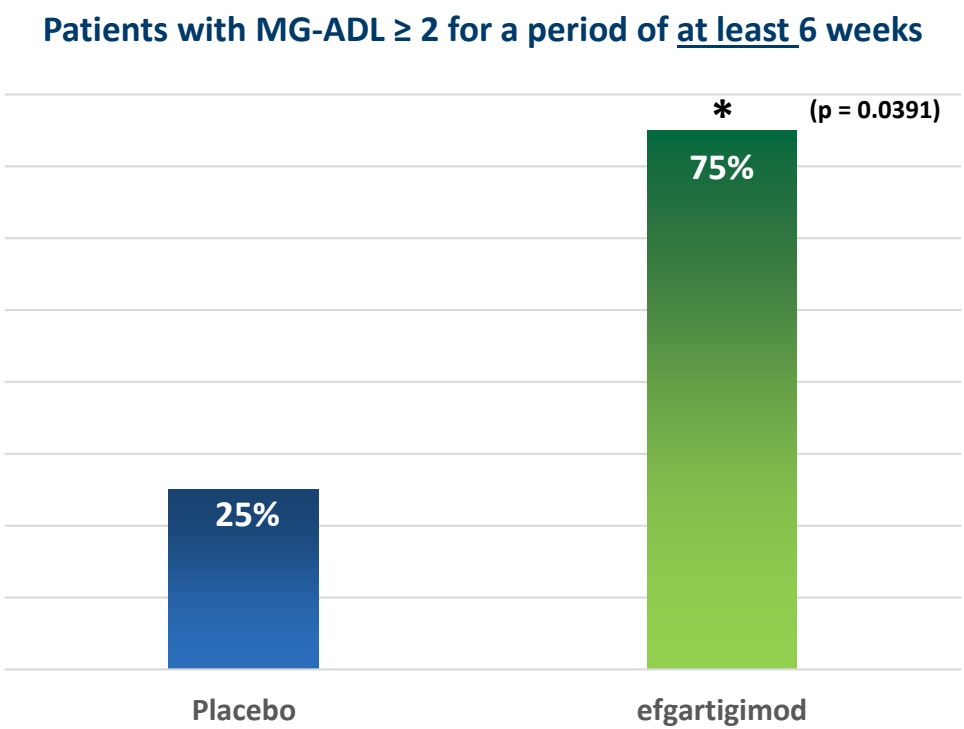
Assessment for all efficacy scales



- 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 (1)

(1) Kuks and Skallebaek, 1998, Transfus Sci

75% of Treated Patients Achieved Lasting Response



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

argenx data

Conclusions Ph2 Study of Efgartigimod in Patients with gMG

- Consistent and compelling safety & tolerability
- Fast, long-lasting and sustained benefit; clinically meaningful and statistically significant
- Strong correlation between IgG level reduction and disease improvement; validating focus on IgG-mediated diseases
- Significant reduction of AChR autoantibodies
- Phase 2 execution accelerates efgartigimod towards Phase 3

Immune Thrombocytopenia (ITP) Overview

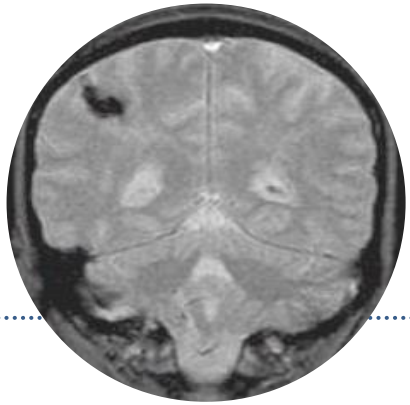
What is Immune Thrombocytopenia?

- Rare bleeding disease:
  estimated 72,000 [(1)] patients in US - ~80% diagnosed with primary ITP
- Symptoms range from mild bruising to severe bleeding
- Symptoms include: mild bruising to severe bleeding, fatigue, fear of bleeding, impact on work and social activities, depression

Limited current treatment options with side effects

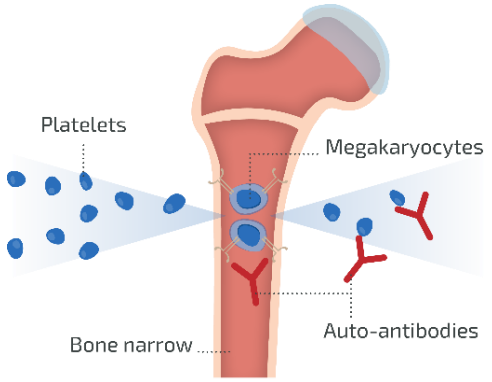
- Multiple iterations on corticosteroids & IVIg
- Immunomodulatory agents
- TPO mimetics & splenectomy

- Romiplostim and Eltrombopag, last-line therapies for ITP and have generated global revenues of $584 million[(2)] and $635 million[(3)] in 2016



Autoantibodies (IgG type):

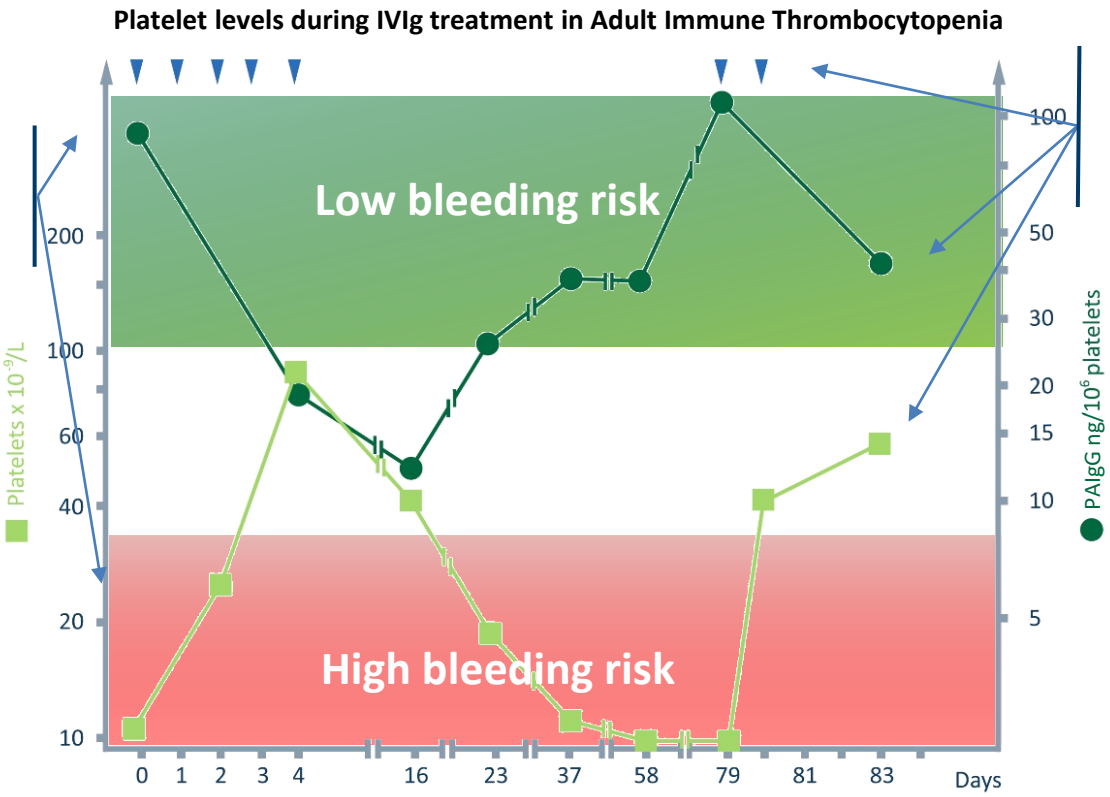
- Enhance platelet clearance
- Kill platelets
- Reduce platelet production
- Inhibit platelet function



(1) Saleh et al. 2015, Curr Med Res Opin.; Terell et al. 2012, Am J Hematol.; Grace et al. 2012, Pediatr Blood Cancer.
(2) Amgen Inc. 2016, Form 10-K.
(3) Novartis Annual Report 2016

Autoantibody Levels (IgGs) Correlate With ITP Disease Score

Autoantibodies inhibit platelet production and accelerate platelet destruction



Therapy aimed at reducing autoantibodies like IVIg (shown), plasmapheresis and immunoadsorption results in platelet increase

▼ = IVIg treatment ● = Autoantibody level ■ = Platelet counts

Winiarski et al. 1983, Scand J Haematol.

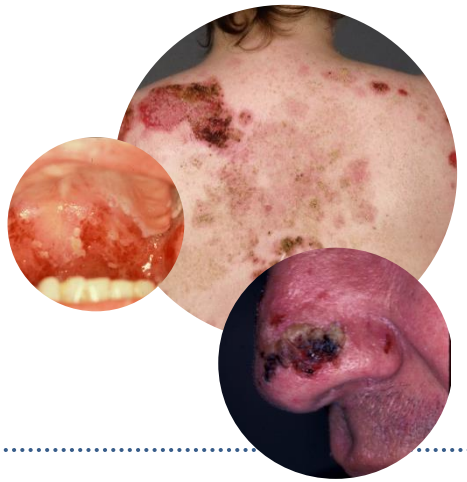
Pemphigus Vulgaris: Overview

What is Pemphigus Vulgaris?

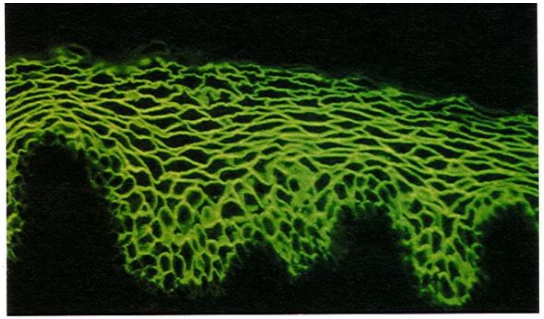
- Chronic, severe auto-immune disease
- 30,000 – 40,000 pemphigus patients (US)[1]
- Mucosal and skin blisters
- Disease severity directly correlates to pathogenic IgG levels against desmoglein-1 (skin) and desmoglein-3 (mucosal)[2]
- Remission and relapse for extended periods

Limited current treatment options with side effects

- Corticosteroids and chronic immunosuppression
- Rituximab, IVIg, immunoadsorption and plasma exchange used for severe or refractory patients (10%), but not curative
- Rituximab therapy shows slow onset of action, risk of developing serious adverse events and significant relapse rate [2] [3] [4]

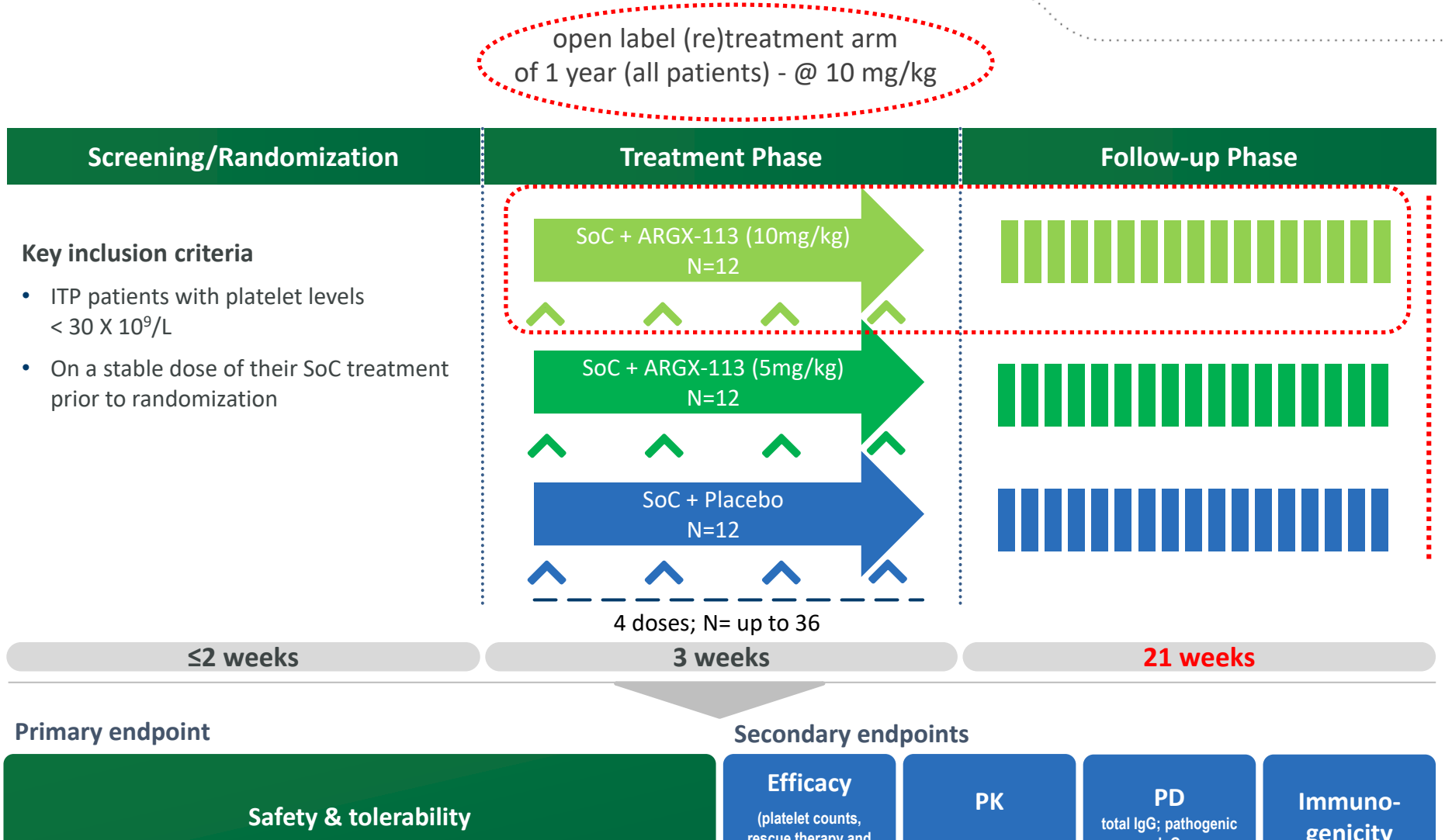


Diagnosis based on presence of pathogenic autoantibodies targeting desmoglein-1 and -3 in the skin

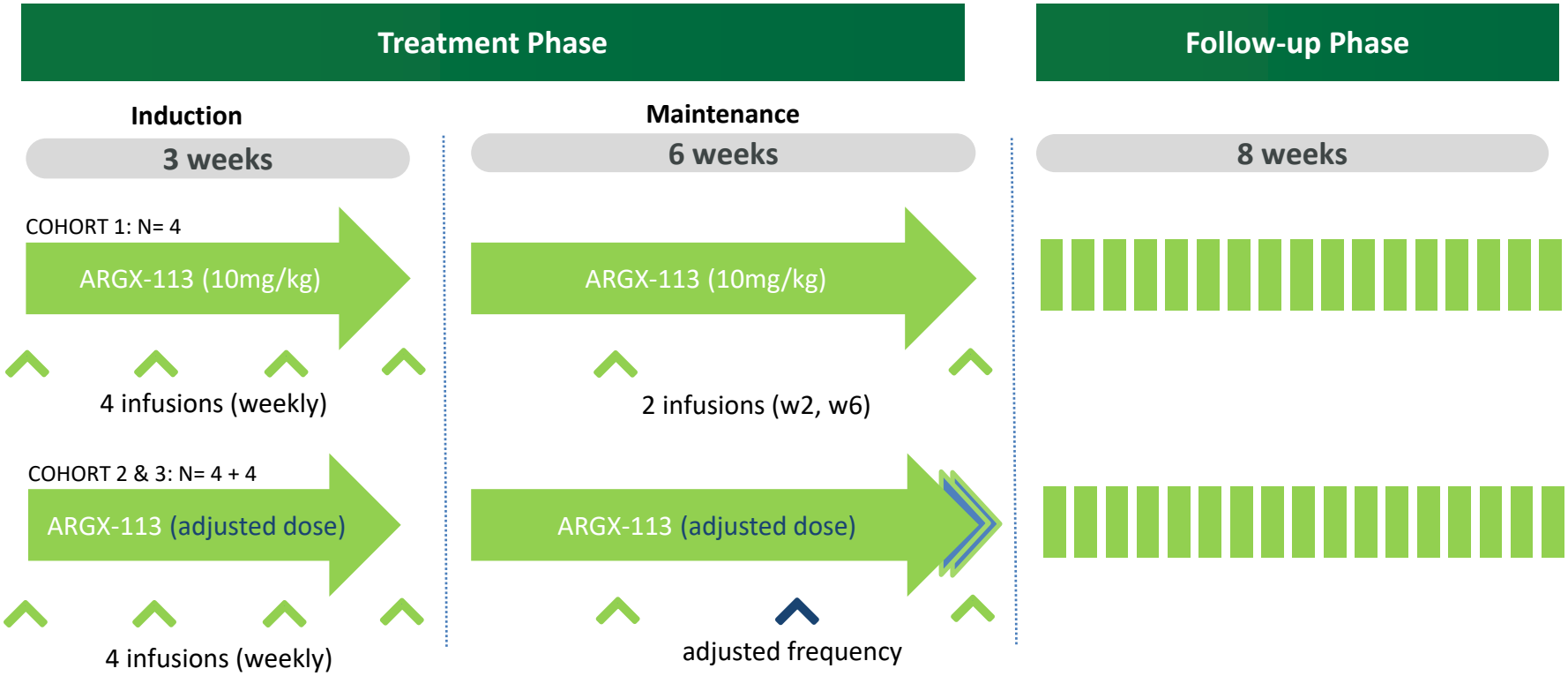


(1) IPPF (2) Kasperkiewicz et al. 2017, Nature Reviews
(3) Colliou et al. 2013, Autoimmunity (4) Joly et al. 2017, Lancet

Immune Thrombocytopenia Phase 2 Amended Trial Design



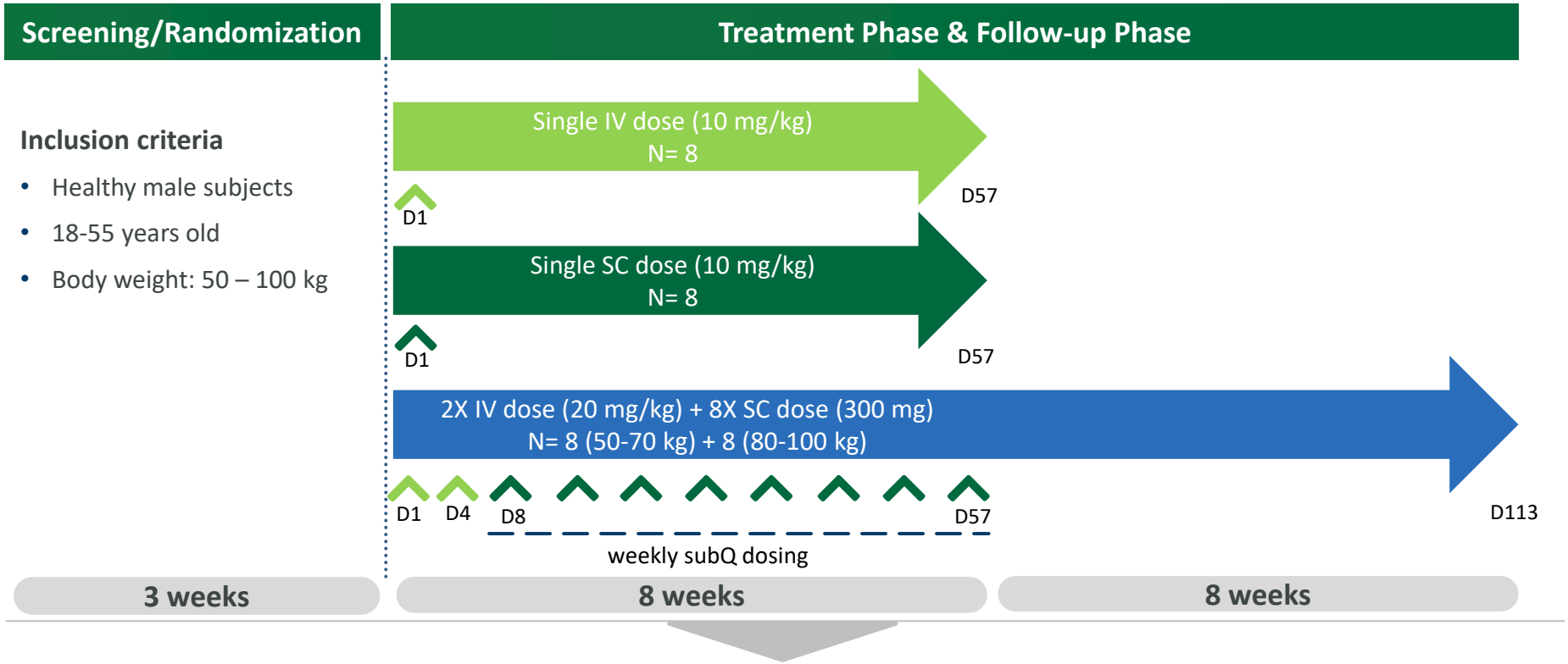
Pemphigus Vulgaris Phase 2 Adaptive Design



- Cohort 1: 10 mg/kg, induction = 4 infusions (3 weeks), maintenance = 2 infusions (6 weeks)
- Additional cohorts:
  - Dose up (25mg/kg) or down
  - Change frequency of dosing at maintenance (up to 2 re-doses)
  - Extend maintenance duration

Phase 1 Healthy Volunteer Subcutaneous Formulation

Open Label Trial Design

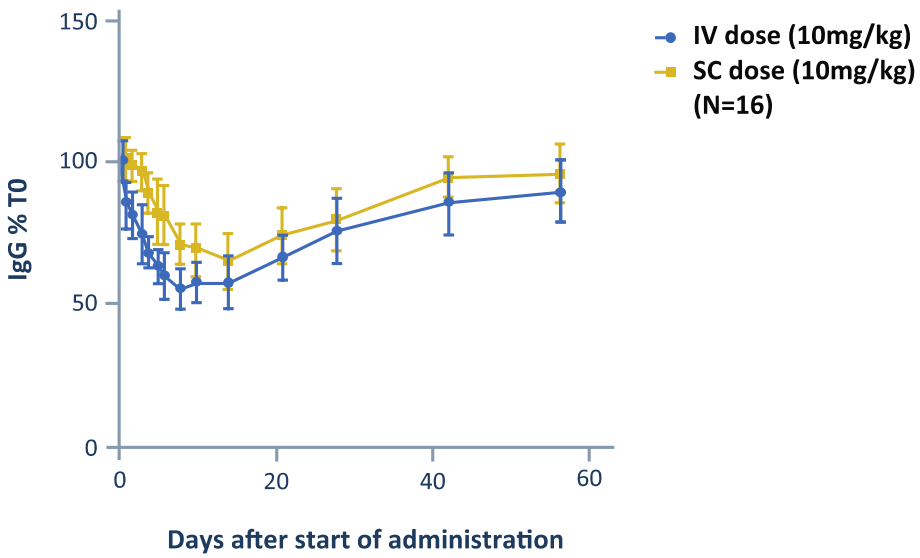


Clinicaltrials.gov: NCT03334084, argenx data

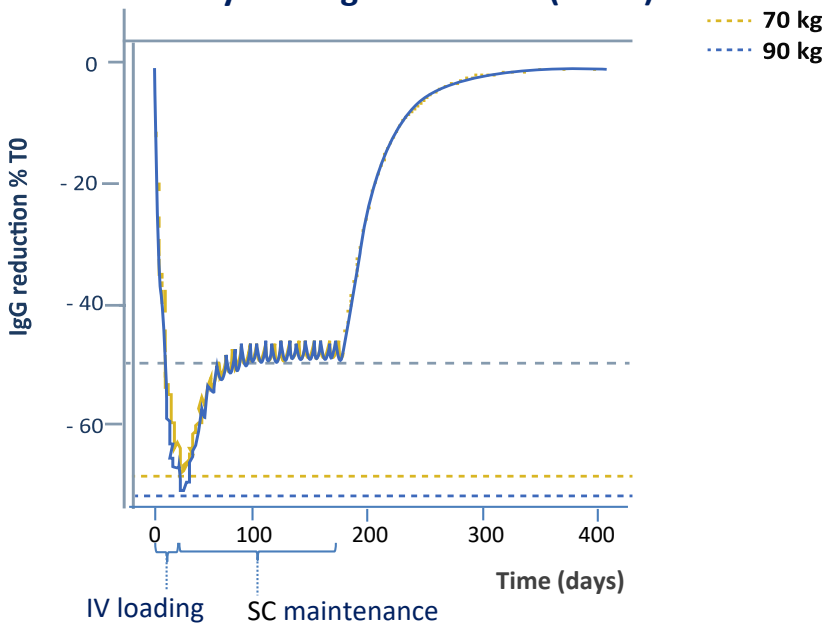
Efgartigimod: Feasibility of Subcutaneous Dosing

Providing optionality to patients

Comparable IgG reduction



Steady state IgG reduction (~50%)



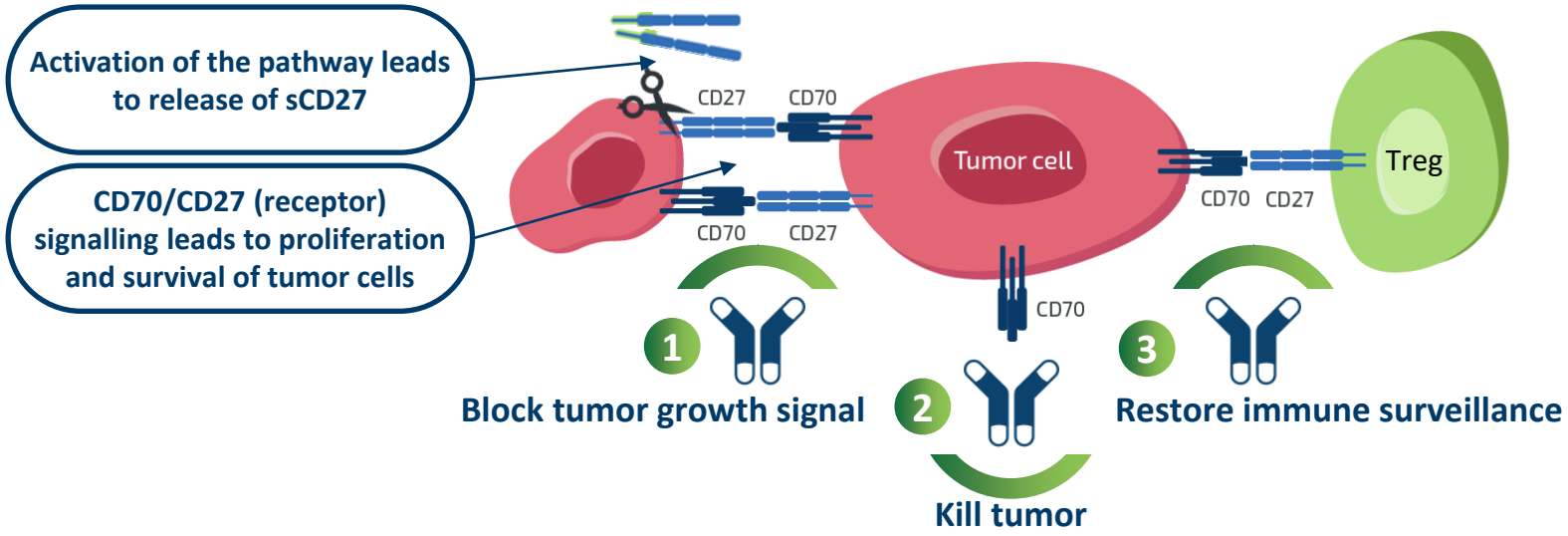
- Viability of SC formulation demonstrated:
  - Comparable half-life to IV
  - Comparable IgG reduction to IV; steady state 50% IgG reduction achieved by weekly dosing (300 mg fixed dose)
  - Favorable bio-availability (~ 50%)
  - Favorable viscosity and stability profile

argenx uncleaned data

ARGX-110: Phase 1 / 2 Mono & Combo Therapy

ARGX-110: Lead Cancer Program Based On Novel Target CD70

Three distinct modes of action to target CD70+ tumor cells



- ARGX-110 is a SIMPLE Antibody™, equipped with POTELLIGENT® Fc engineering technology
- ARGX-110 targets CD70 to block CD27 interaction, kill CD70 expressing cells and restore immune surveillance
- Soluble CD27 is a biomarker
- Phase 1: encouraging safety & tolerability profile and promising preliminary signs of efficacy in CTCL
- Focus on two rare & aggressive hematological tumors: CTCL and newly diagnosed AML / high-risk MDS
  - Interim results from dose escalation part of Phase 1/2 AML/MDS trial expected YE:2017
  - Interim POC data from Phase 2 CTCL trial expected YE:2017

Silence et al. 2014, mAbs.

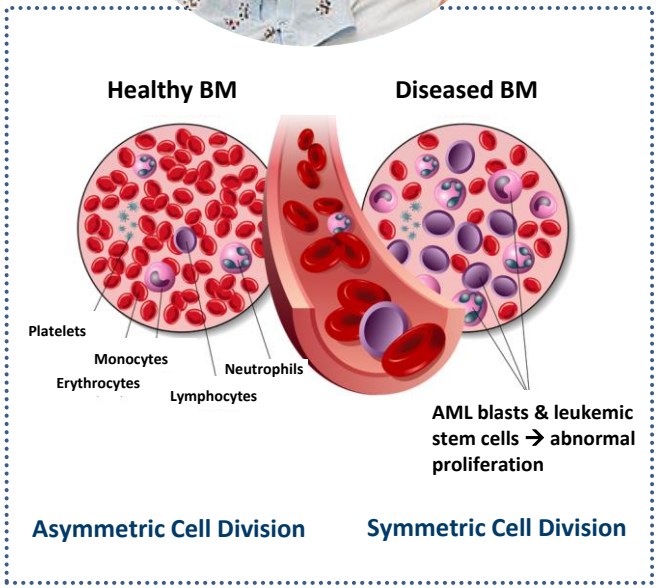
Acute Myeloid Leukemia (AML) Overview

What is Acute Myeloid Leukemia?

- Rare hematologic cancer characterized by excessive proliferation of myeloid stem cells and their failure to properly differentiate into mature white blood cells
- AML progresses very rapidly and is fatal if left untreated
- ~22,000[(1)] new cases per year in the U.S.
- Disease of the elderly — 60% of diagnosed patients are older than 60yr

Limited current treatment options

- Elderly, frail patients unfit for high dose chemotherapy — palliative treatment with hypomethylating agents
  - Median survival of 7 – 10 months
  - ~6%[(2)] five year survival rate for patients over 65
- First-line treatments for patients <45yr: aggressive chemotherapy followed by stem cell transplant
  - 5-year survival is ~57%[(2)] for patients under 45

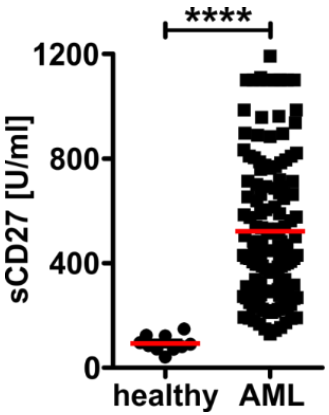


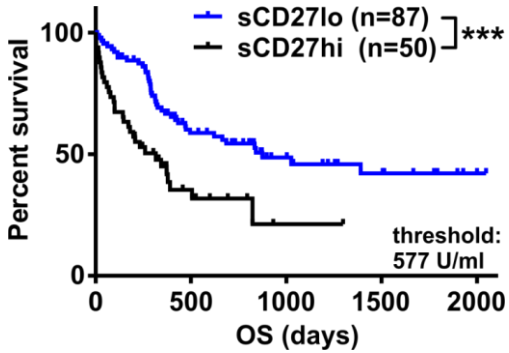
(1) American Cancer Society: http://www.cancer.org/cancer/leukemia-acutemyeloidaml/detailedguide/leukemia-acute-myeloid-myelogenous-key-statistics .
(2) National Cancer Institute: Howlader et al. (eds). SEER Cancer Statistics Review, 1975-2013, National Cancer Institute. Bethesda, MD, http://seer.cancer.gov/csr/1975_2013/, based on November 2015 SEER data submission, posted to the SEER web site, April 2016. Table 13.16

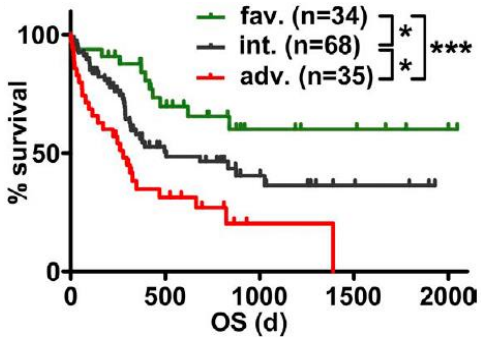
CD70 Provides Unifying Rationale Across Risk & Age Classes In AML

Potential to selectively target leukemic stem cells in AML patients

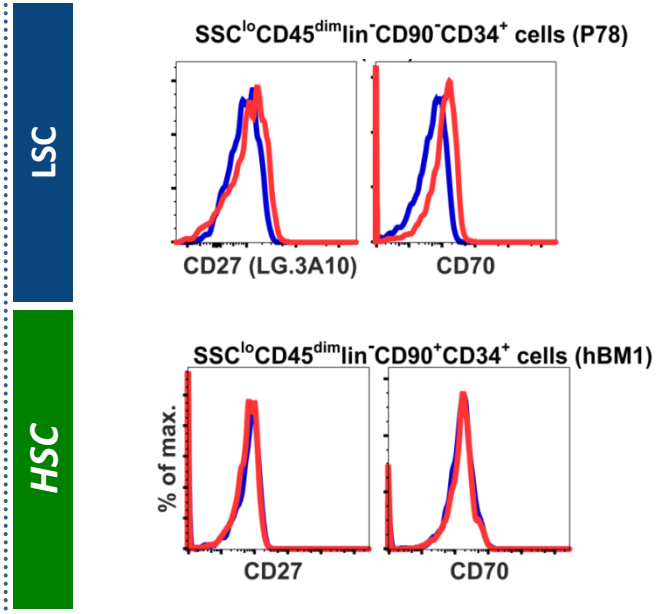
Elevated sCD27 serum levels correlate with poor prognosis







CD70 is a selective LSC marker



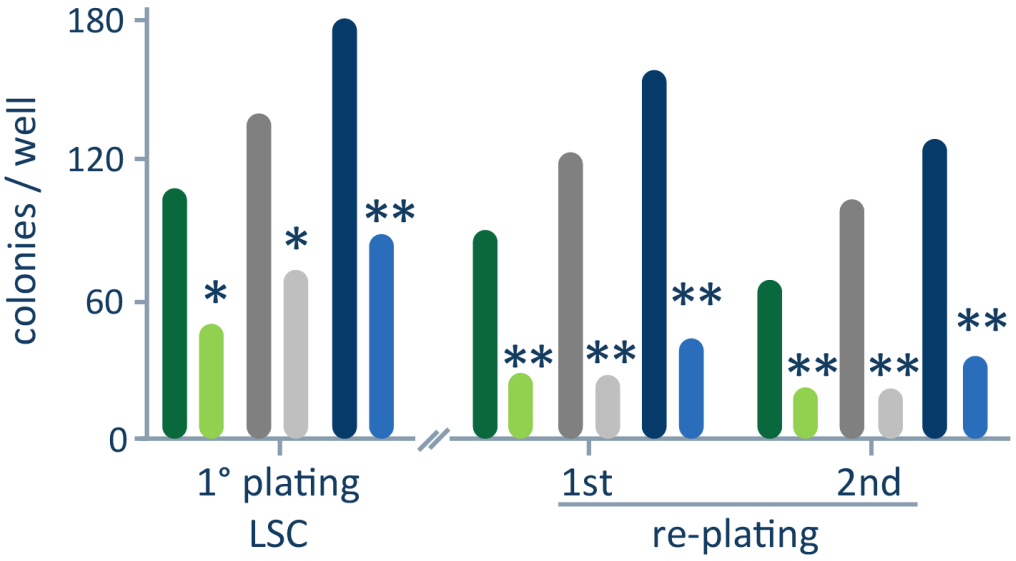
Legend: adv., adverse; CI, confidence interval; fav., favorable; int., intermediate; OS, overall survival. Statistics: left: one-way ANOVA; middle: log-rank test. *, P < 0.05; **, P < 0.01; ***, P < 0.001.

- Elevated sCD27 serum levels in all newly diagnosed AML patients, regardless of risk or age categories
- sCD27 levels are an independent negative prognostic marker in all newly diagnosed AML patients
- CD70 expressed on ~86-100% of AML blasts, majority of malignant cells are CD70/CD27 double-positive
- CD70/CD27 selectively overexpressed on leukemic stem cells (LSCs), not on hematopoietic stem cells (HSCs)

Riether et al. 2017, J Exp Med.

ARGX-110: Inhibits LSC Proliferation In Lasting Fashion

Long-term effects *ex vivo*



After o/n incubation colony assays (14d)
+ARGX-110 variant

Bulk cells from 1st colony

Serial re-platings
-ARGX-110 variant

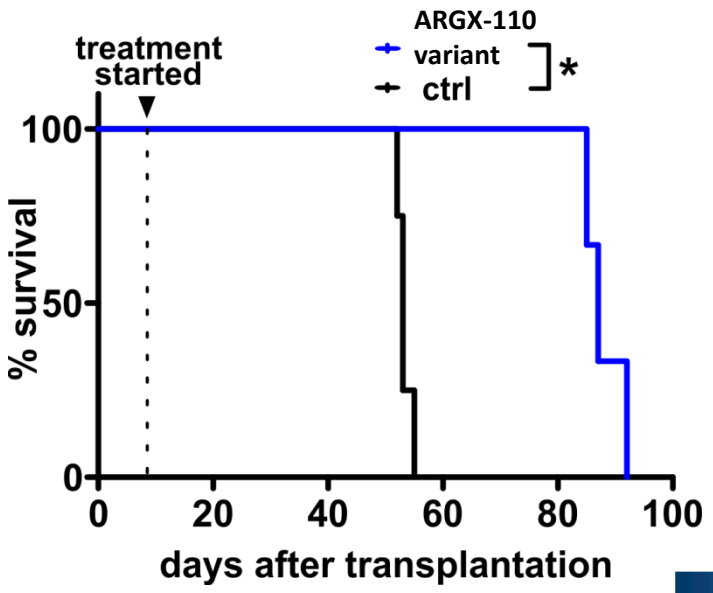
ARGX-110 variant = ARGX-110 without Fc effector functions

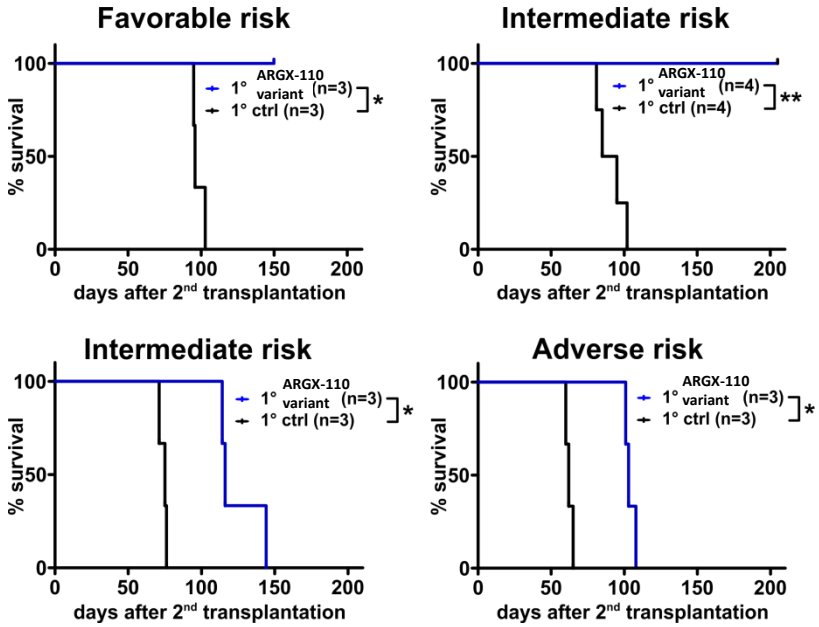
- Reduces LSC colony formation across patient risk categories (favorable/intermediate/adverse risk)
- Reduces LSC numbers as determined in serial re-plating experiments
- Blocking CD70 results in: (1) lasting down-regulation of stem cell genes (2) increasing myeloid differentiation

Riether et al. 2017, J Exp Med.

ARGX-110: Curative Potential Of Monotherapy In Mouse Model

Shown to reduce LSCs, increasing survival in AML model





Initial in vivo treatment
+ARGX-110 variant

Grafting Whole Bone Marrow cells from treated into new mice (14d after start of treatment)

Transplant, evaluation
-ARGX-110 variant

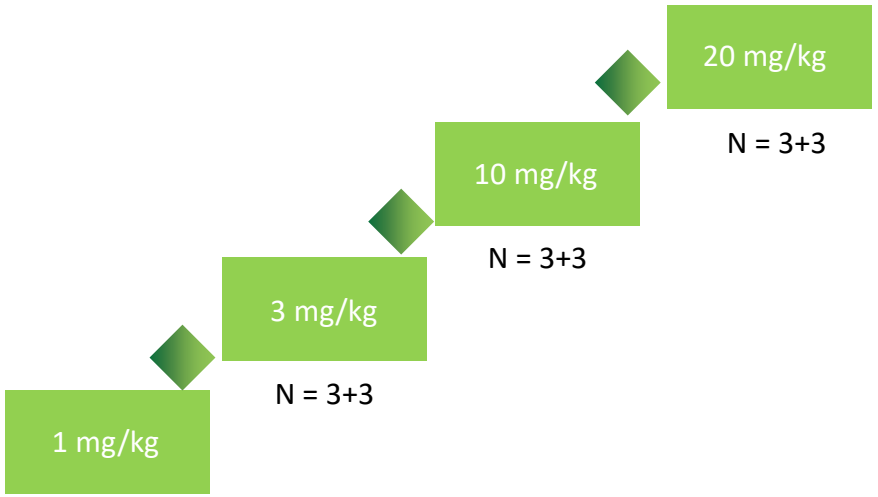
- Increased survival after secondary transplantation of AML BM cells from primary recipients transiently treated with ARGX-110 variant
- Increased survival observed for AML blasts taken from all 3 AML risk categories (fav/int/adv)

Riether et al. 2017, J Exp Med.

ARGX-110 & Azacitidine For AML/MDS: Phase 1 / 2 Combo

Phase 1 – Dose escalation

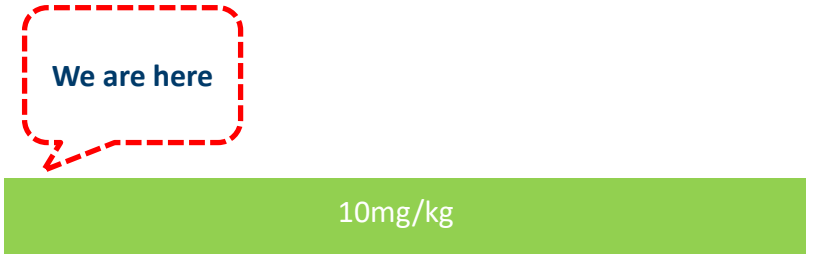
Safety and tolerability



Vidaza = 75 mg/m^2 (standard of care)

N = up to 24

Phase 2 – Proof of Concept



Vidaza = 75 mg/m^2 (standard of care)

N = 21

- Hypomethylation agents such as Azacitidine increase CD70 expression[1]
- Population: untreated AML & high risk of myelodysplastic syndrome (MDS)*, eligible for AZA
- Design: open-label, non-controlled, non-randomized

*Some Myelodysplastic Syndrome (MDS) patients are at high risk of developing AML; MDS affects bone marrow cells, reducing their ability to produce red & white blood cells

(1) Zhou et al. 2011, Lupus.

Non-Transplantable Patients With Intermediate & Adverse Risk and High Blast Count in Bone Marrow

9 newly diagnosed AML patients

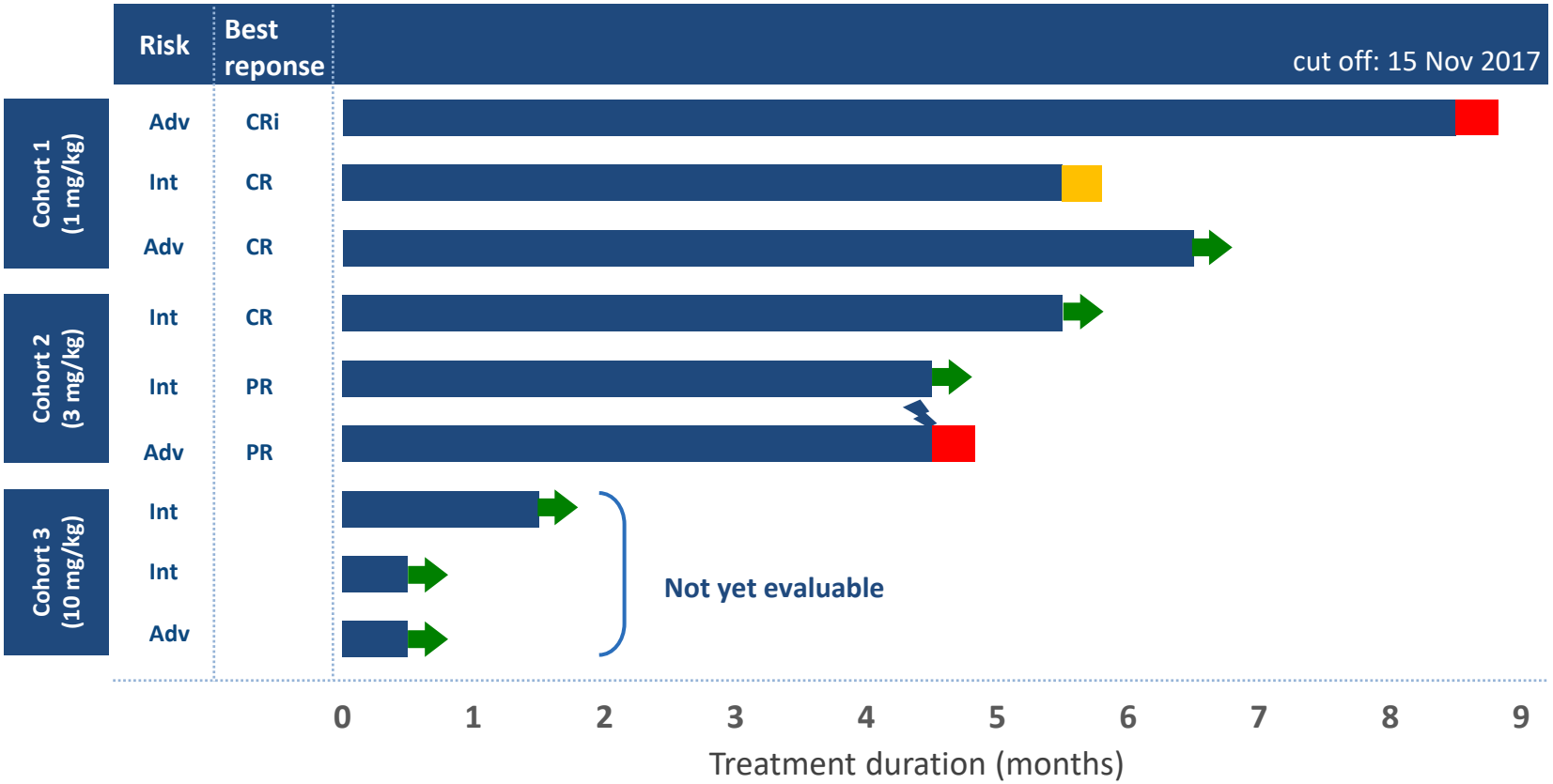
Baseline characteristics (N=9)	ARGX-110 + Azacitidine			
	1 mg/kg	3 mg/kg	10 mg/kg	Total
Age				
Median	71 71-80	75 71-84	71 64-75	**72** **64-84**
Gender: Male/Female	2/1	1/2	2/1	**5/4**
Risk (ELN 2017)				
Intermediate	1	2	2	**5**
Adverse	2	1	1	**4**
Blasts in the bone marrow				
Median %	51.3 24-90	40 20-60	70 50-80	**53.6** **20-90**
AML classification (WHO 2016)				
Not other specified		1	3	**4**
With Myelodysplasia- related changes	2	2		**4**
Therapy-related myeloid neoplasm	1			**1**
French-American-British subtypes	M4,M1,M2	M4,M5,M2	M1,M2,M5a	

ELN: European Leukemia Net, Dohner et al. 2017, Blood

Source: argenx data – patient anecdotes – uncleaned data

Response in 6/6 Evaluable Newly Diagnosed AML Patients

ARGX-110/Aza treatment



- So far, all patients responded (3 CR, 1 CRi, 2 PR)
- 1 patient reached CR and bridged to allogeneic stem cell transplant after 5 cycles
- 6/9 patients were still on treatment

Study ended

Patient successfully transplanted

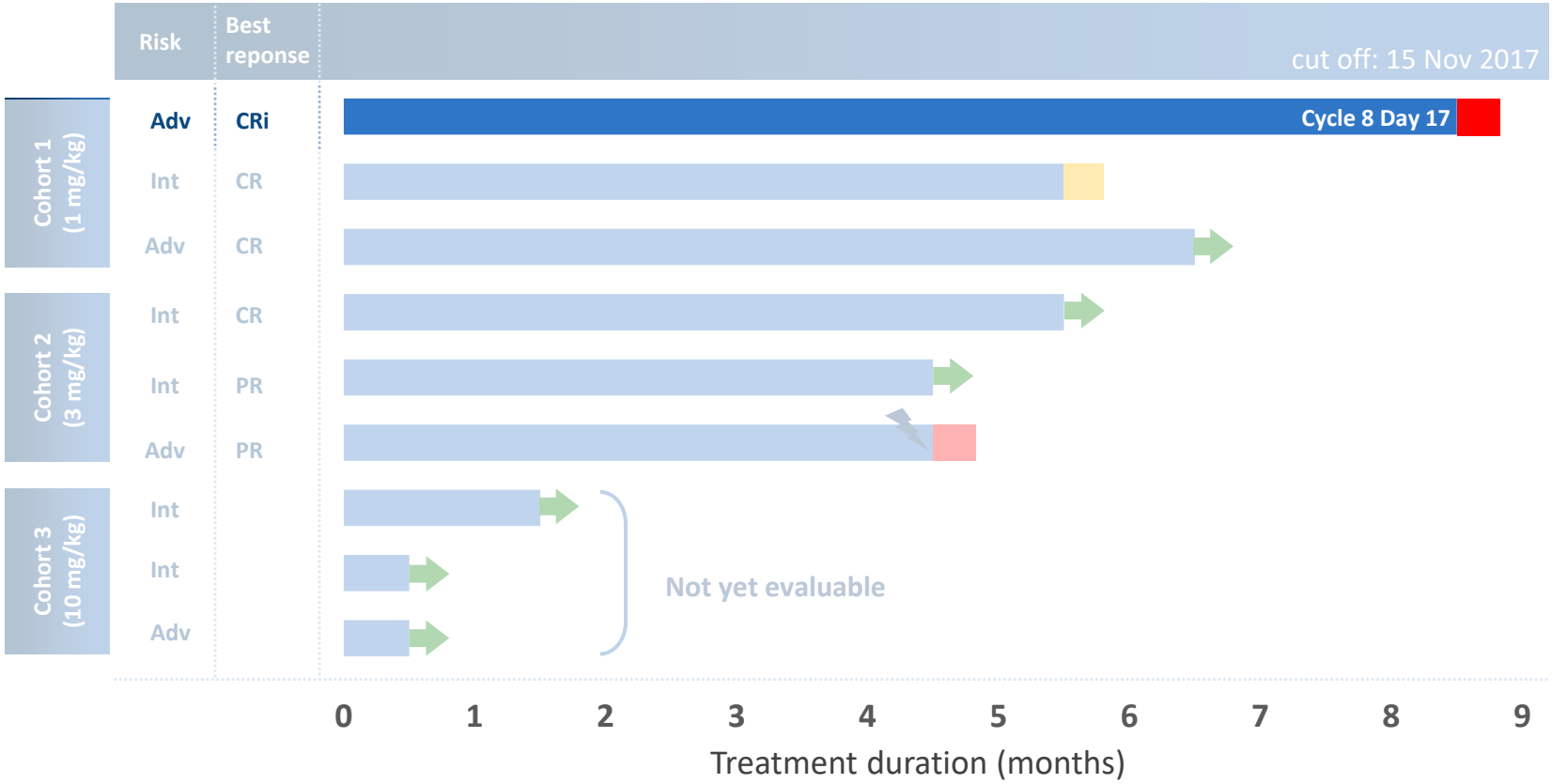
Adverse event leading to discontinuation

Ongoing study

Source: argenx data – patient anecdotes – uncleaned data – presented at ASH (Dec 11, 2017)

Case 1: Patient Cohort 1 – 1 mg/kg – 8 Cycles on Study

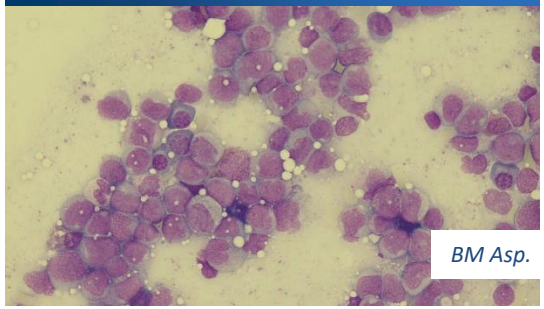
- 80 year old female
- Therapy-related AML, M4; BM ~65% blasts
- Molecular genetics: FLT3-ITD; DNMT3Amut; RUNX1mut; WT1mut; cytogenetics: normal



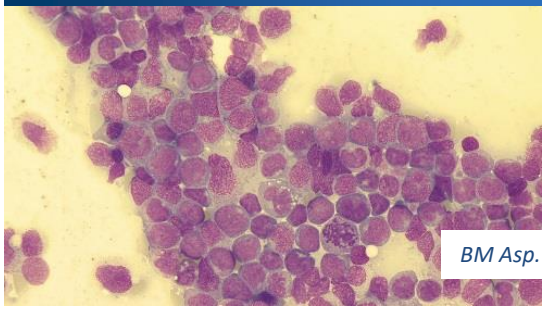
Source: argenx data – patient anecdotes – uncleaned data

Case 1: Complete remission with incomplete hematological recovery

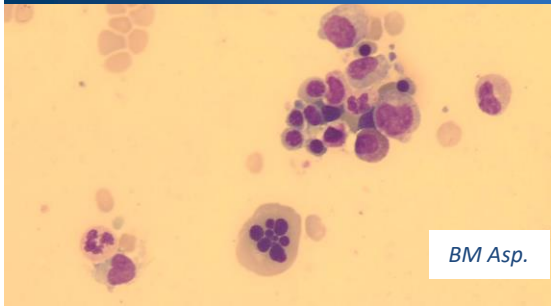
Screening



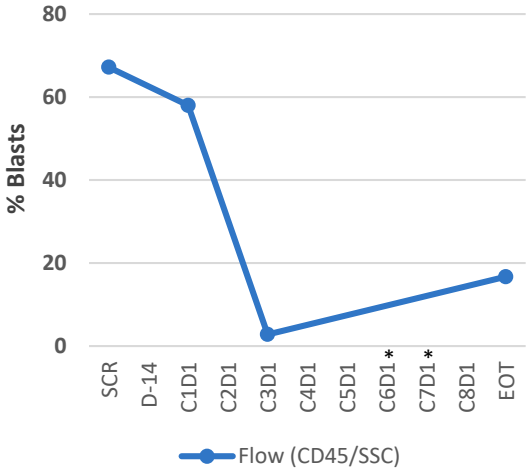
Leukemic blast persistence – (C1D1)



Leukemic clearance – (EOT)



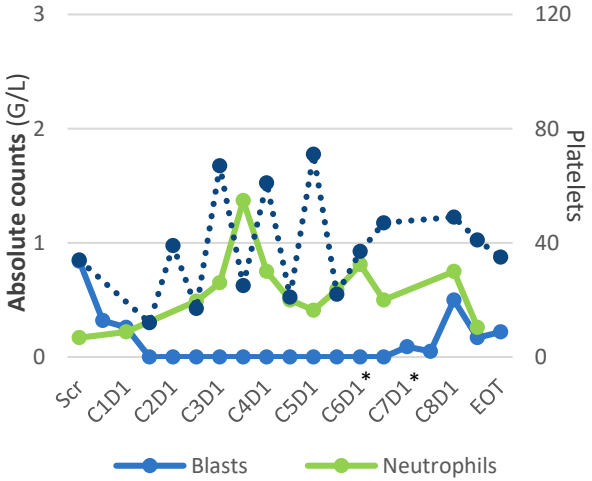
Bone marrow: % Blasts, flow cytometry



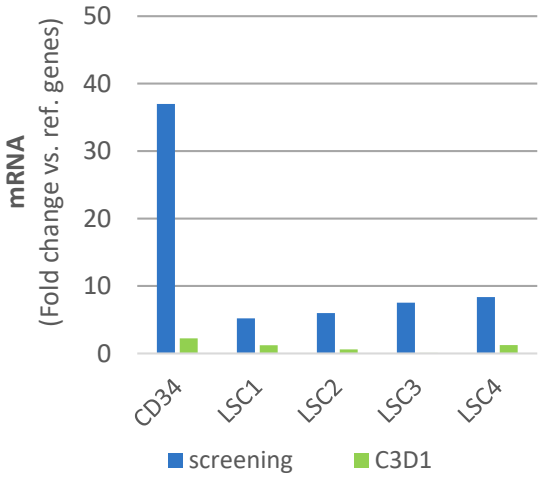
* Drug holiday (cfr Azacitidine toxicity)

Ng et al. 2016, Nature

Blood analysis: Absolute counts (G/L)



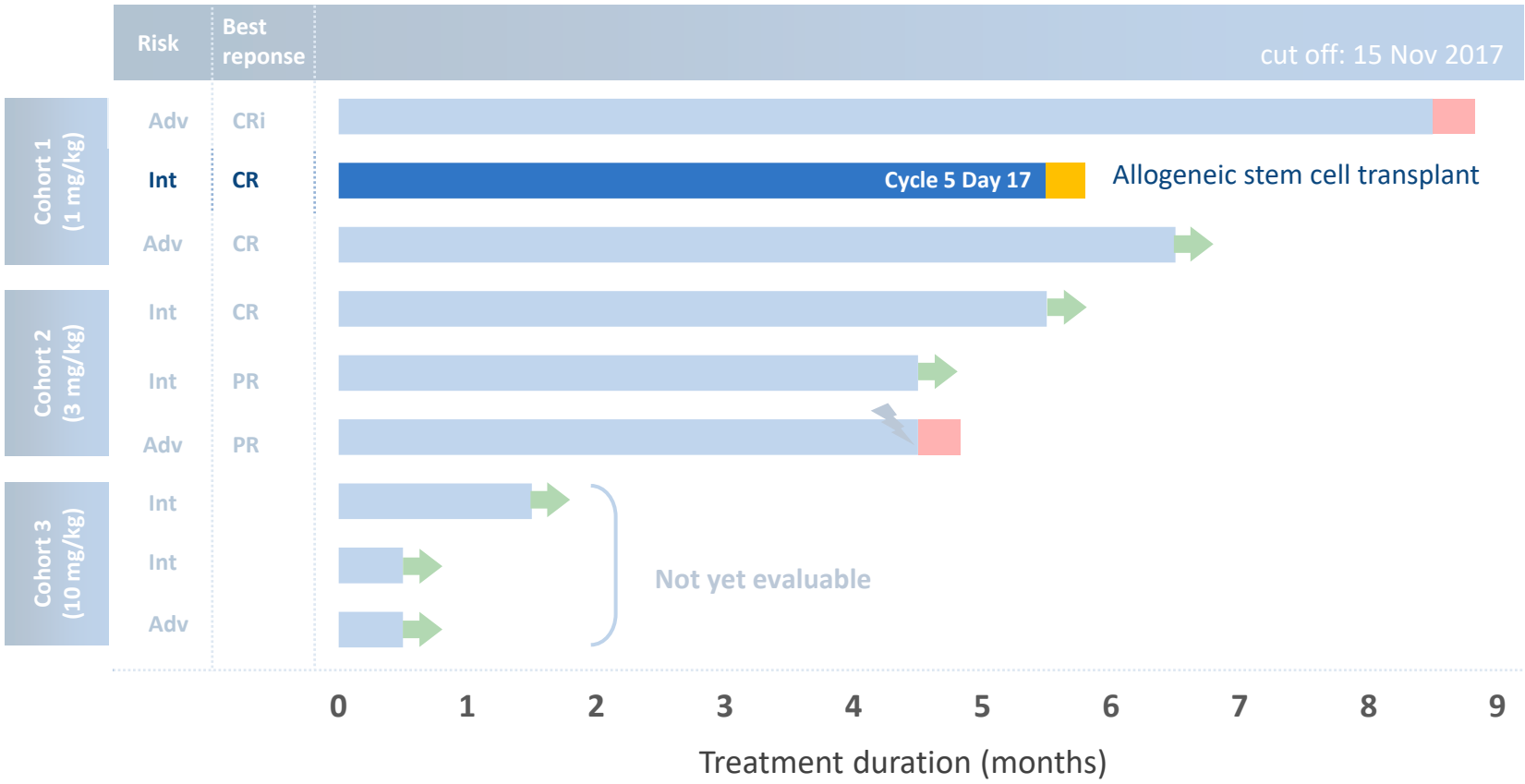
ARGX-110/Aza reduces experimental LSC gene signature



Source: argenx data – patient anecdotes – uncleaned data

Case 2: Patient Cohort 1 – 1 mg/kg – 5 Cycles on Study

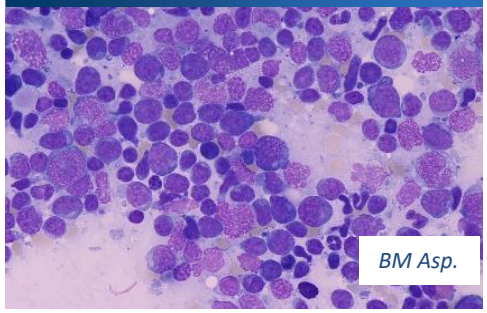
- 75 year old male
- AML with myelodysplasia-related changes, M1/M2; BM ~40% blasts
- Molecular genetics: U2AF1mut; DNMT3Amut; cytogenetics: normal



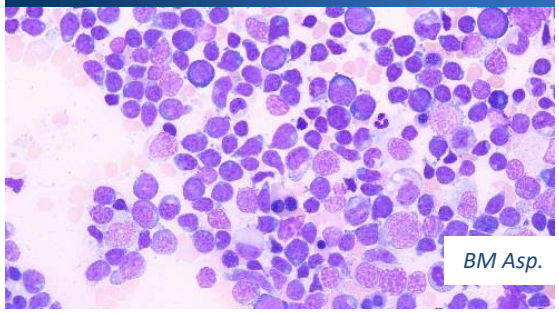
Source: argenx data – patient anecdotes – uncleaned data

Case 2: ARGX-110/Aza Induces Complete Remission & Bridges to Transplant

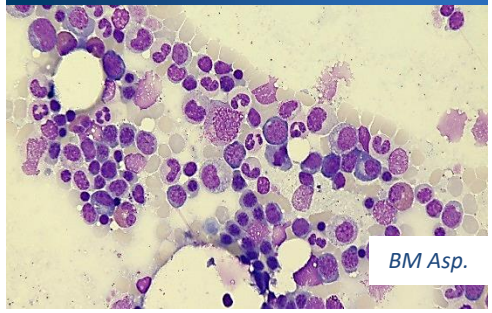
Screening



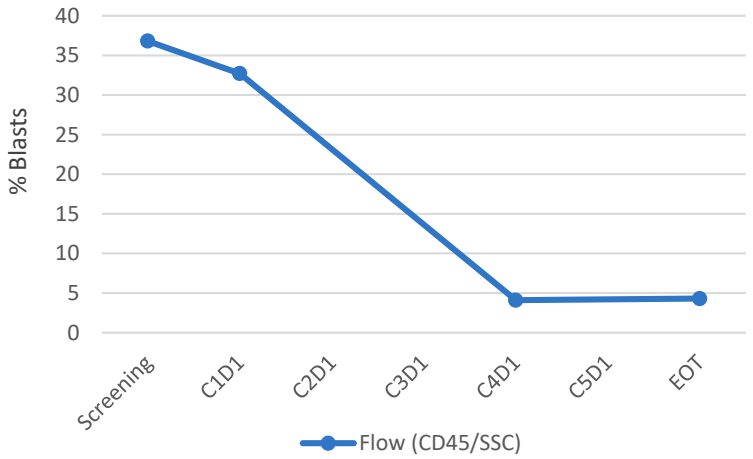
Leukemic blast persistence – (C1D1)



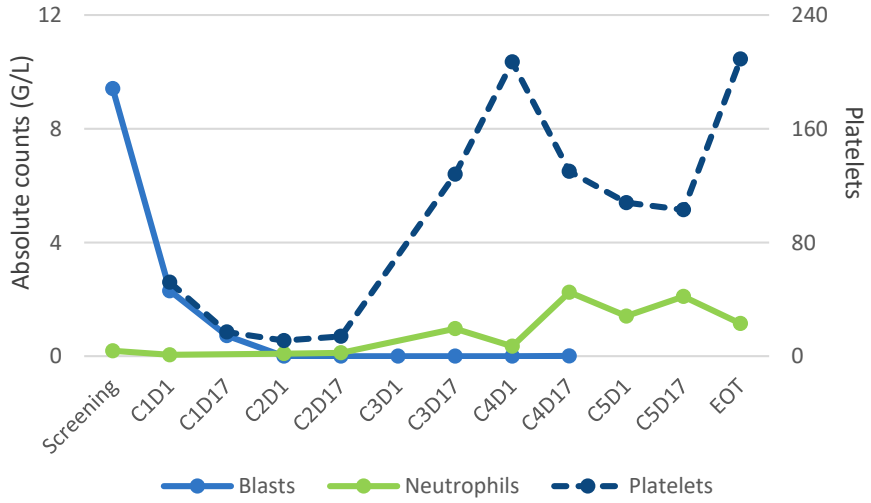
Leukemic clearance – (EOT)



Bone marrow: % Blasts, flow cytometry



Blood analysis: Absolute counts (G/L)



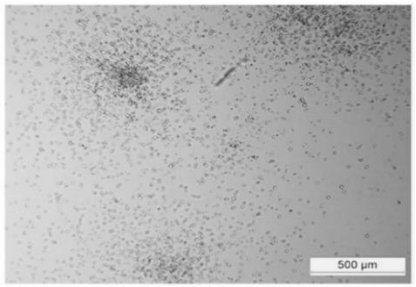
Source: argenx data – patient anecdotes – uncleaned data

Case 2: ARGX-110/Aza combo reduces AML stemness

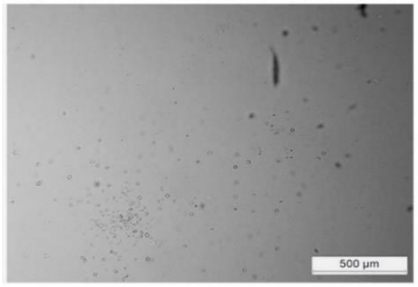
ARGX-110 monotherapy reduces LSCs outgrowth

White light microscopy (5,000 cells)

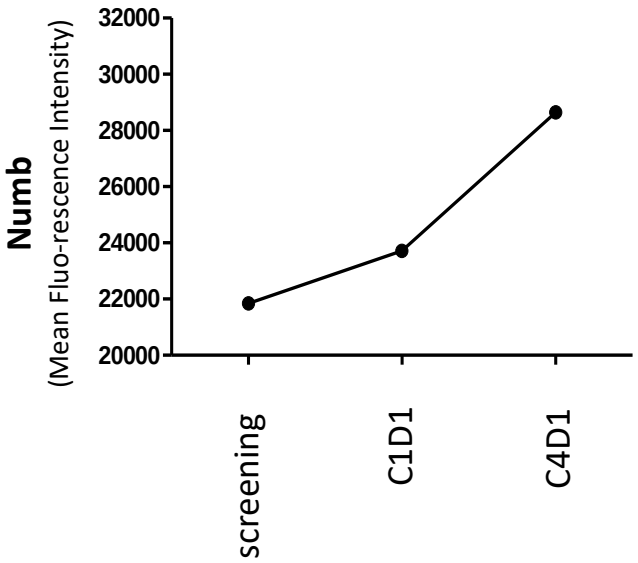
Screening



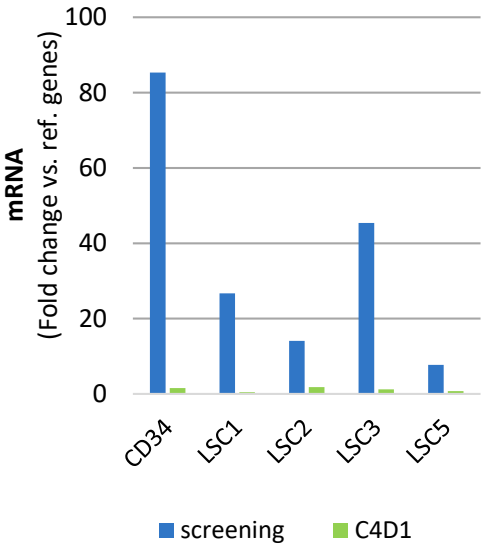
ARGX-110: Cycle 1 Day 1



ARGX-110/Aza increases asymmetric LSC division



ARGX-110/Aza reduces experimental LSC gene signature



'It seems ARGX-110 targets mature blasts as well as LSCs – this is very promising' (AML KOL)

- Significantly reduced leukemic stem cell colony formation
- Increased myeloid differentiation (asymmetric division) of leukemic stem cells
- Reduction of LSC gene signature

Ng et al. 2016, Nature

Source: argenx data – patient anecdotes – uncleaned data

ARGX-110 In Newly Diagnosed AML Patients – Summary

Preliminary data from first 6 patients – additional data needed

Preliminary clinical data confirm preclinical observations

Promising preliminary activity obtained in first set of patients

- 6/6 responders
- 1 patient bridged to transplantation

Encouraging safety and tolerability profile

- No exacerbation of azacitidine toxicity

Highly differentiated drug profile

- CD70 uniformly & selectively expressed
- Driving LSCs into myeloid differentiation

'In an ideal world, a LSC targeting drug should show response regardless of risk category, should show a better response in de-novo vs R/R patients and should allow for deep and durable responses. ARGX-110 may meet these criteria' (AML KOL)

Source: argenx data – patient anecdotes – uncleaned data

Business development & financials

AbbVie Partnership for Novel Target GARP

Strategic Antibody Collaboration Details

- **GARP** is a protein specifically present on the surface of activated regulatory T-cells (Tregs)
- **AbbVie** has option to:
  - Obtain exclusive, worldwide license to develop and commercialize ARGX-115
  - Fund further GARP-related research by argenx beyond ARGX-115
- **argenx** conducts and funds R&D through IND-enabling studies
- **argenx** can study ARGX-115 in combo with its pipeline programs

Financial Highlights

- **$40mm upfront payment**
- **Received** first of two **$10mm** preclinical milestones
- **$625mm in potential** development, regulatory and commercial **milestones**
- **Tiered royalties** on sales at percentages ranging from **mid-single digits to low teens**
- **Co-promotional** rights for ARGX-115-based products in the **European Economic Area and Switzerland**

Cuende et al., 2015, Science Trans Med.

Additional Strategic Collaborations

Partner	Asset	Key commentary
嘉和生物药业 Genor Biopharma	**ARGX-109** (Gerilimzumab)	• Mutually terminated license agreement with Bird Rock Bio • Development for Chinese market
LEO	**ARGX-112**	• Focused on **inflammation-based dermatological indications** • LEO Pharma funds >50% of all development costs up to CTA approval and all development post-approval of first Phase 1 trial in Europe • argenx is eligible for ~€100mm in aggregate milestone payments + tiered royalties
STATEN BIOTECHNOLOGY	**ARGX-116**	• Focused on developing an anti-ApoC3 antibody for **dyslipidemia** • Jointly responsible for conducting dyslipidemia research — Staten responsible for additional clinical development • argenx eligible for royalties in the low twenties
Shire	**Discovery Programs**	• Focused on **novel rare disease targets** • Provides Shire access to SIMPLE Antibody™ platform + Fc engineering technologies • argenx has received $12mm in aggregate upfront and milestone payments and R&D fees over the course of the collaboration • Shire purchased €12mm of argenx ordinary shares through participation in July 2014 IPO

Financial Strength

NASDAQ IPO & follow-on financing in 2017

EVENT	DATE	GROSS PROCEEDS
Euronext – Initial Public Offering	July 2014	€42mm
PIPE	June 2016	€30mm
Nasdaq – Initial Public Offering	May 2017	$115mm (€102mm)
Follow-on	December 2017	$266mm (€226mm)

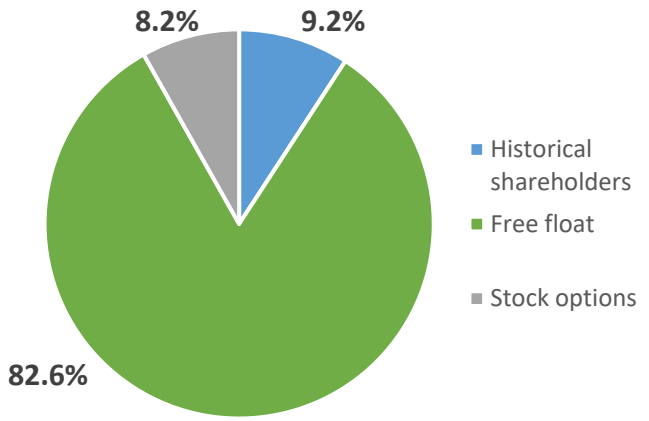
Financial Profile and Investor Composition

Shareholder base > 60% US investors

Additional Key Statistics – March 31, 2018

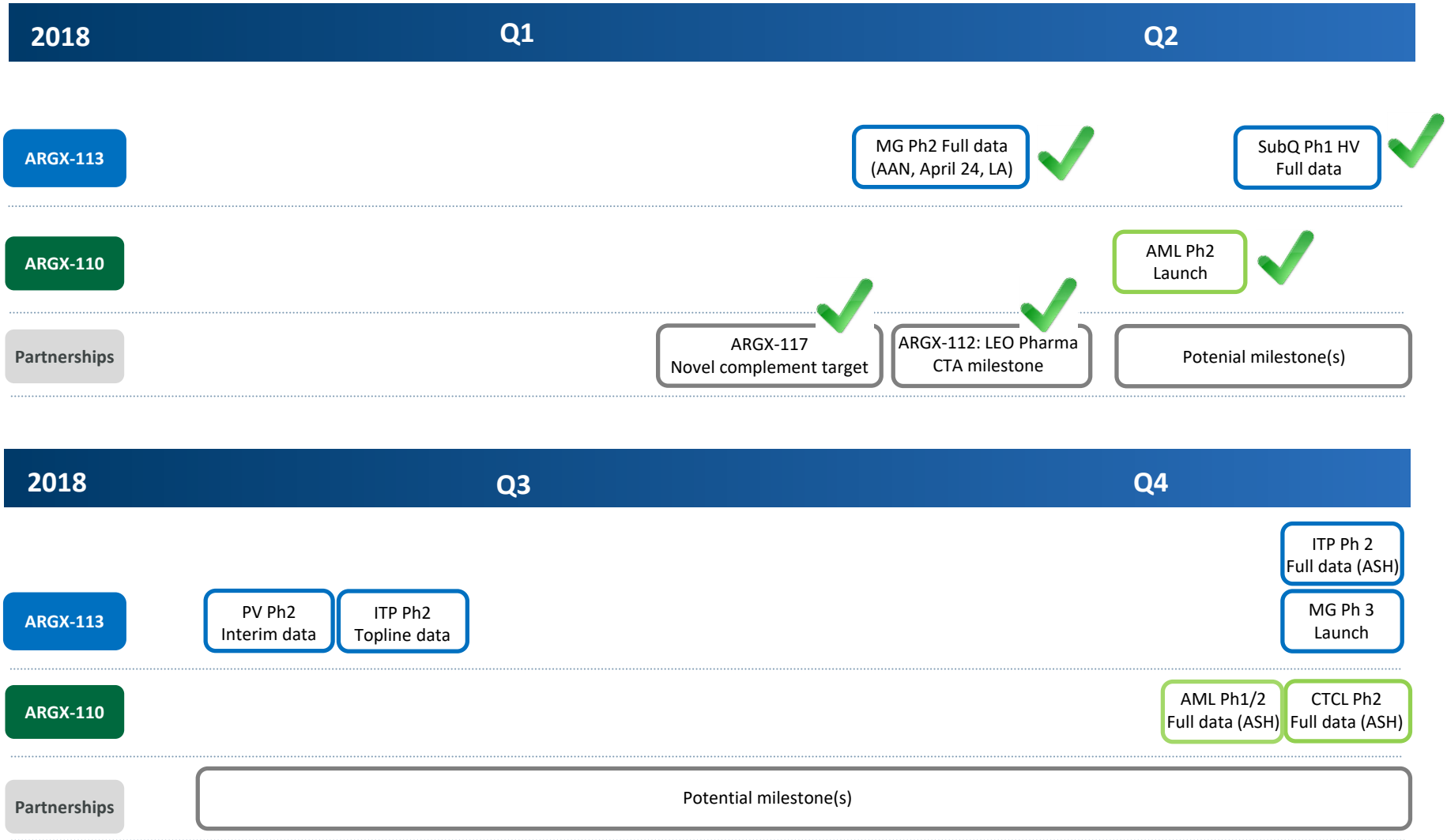
- **Cash position:** €347mm
- **Capital raised since inception: €475mm (ex. grants)**
  - 2017: raised $115mm (€102mm) in NASDAQ IPO
  - 2017: raised $266mm (€226mm) in public offering
- **Non-dilutive funding since inception: €91mm (incl. grants)**
  - 2017: $10mm preclinical milestone AbbVie
- **104 employees & consultants —80 R&D, 24 SG&A**

Blue-Chip Investor Base – Febr, 2018



- **US shareholding** expanded **above 60%**

Key Upcoming Milestones & Communications



Appendix

Company Leadership

Management

Tim Van Hauwermeiren
Chief Executive Officer

Keith Woods
Chief Operating Officer

Hans de Haard, Ph.D.
Chief Scientific Officer

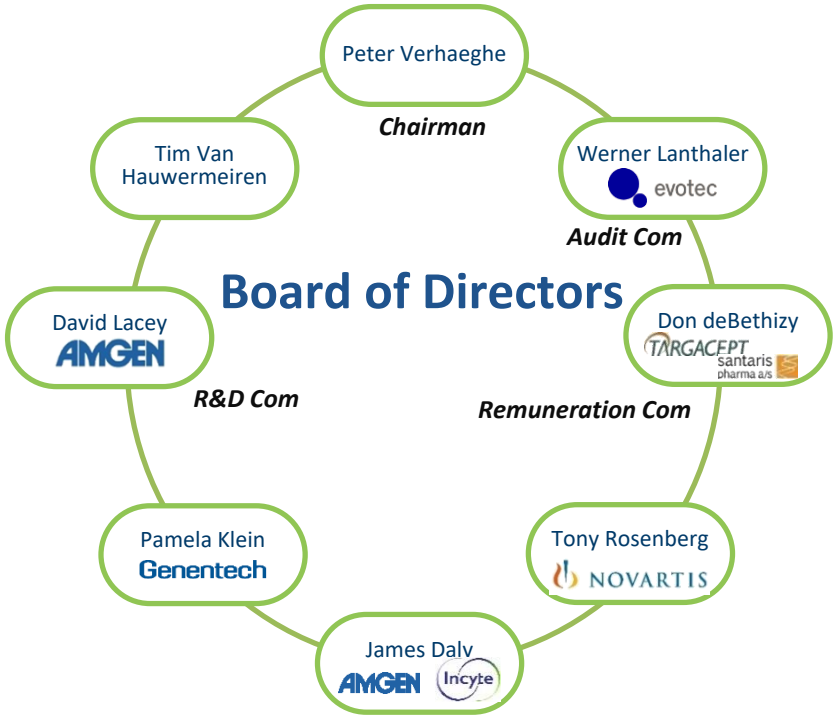
Torsten Dreier, Ph.D.
Chief Development Officer

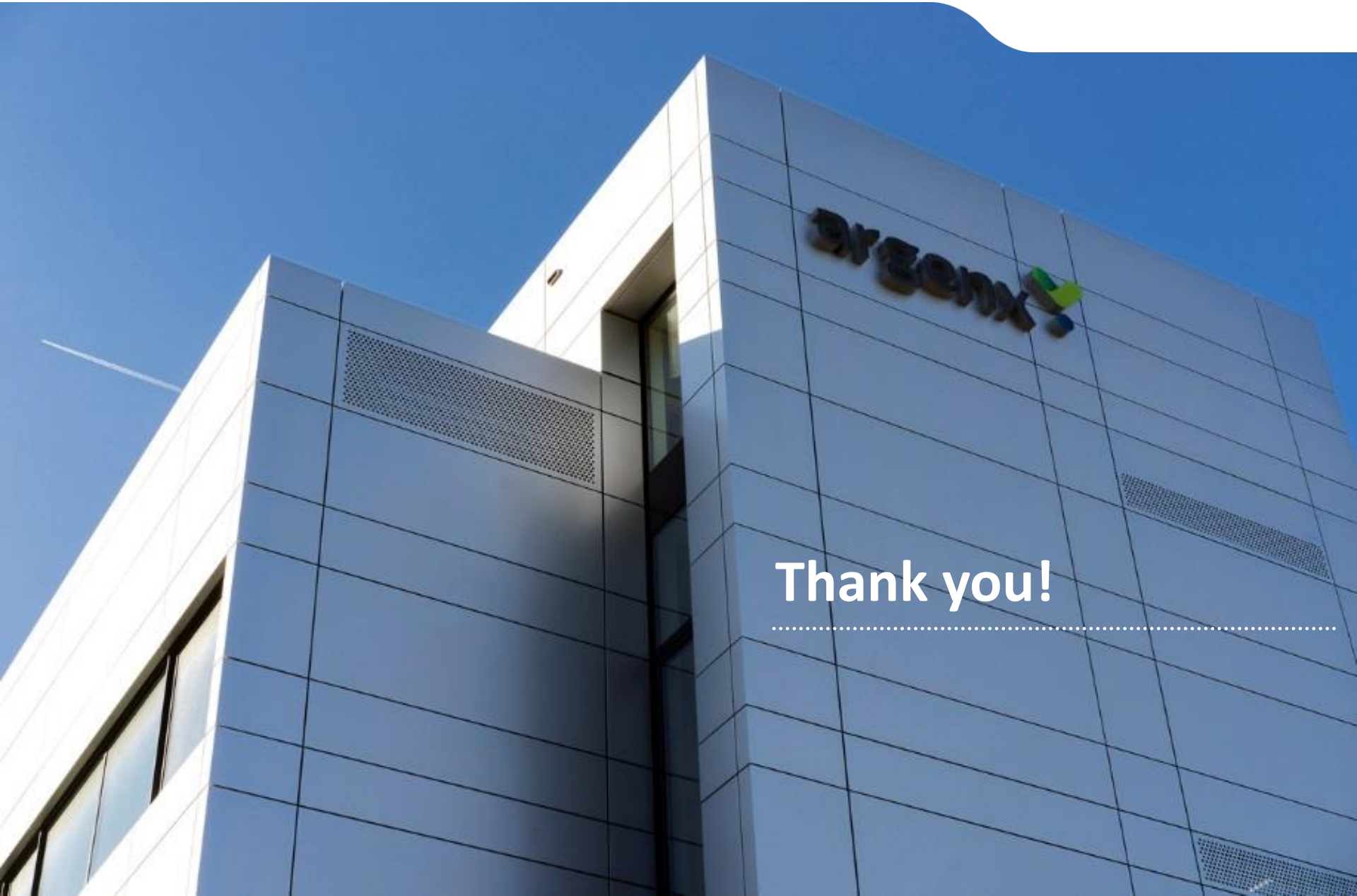
Eric Castaldi
Chief Financial Officer

Nicolas Leupin, M.D.
Chief Medical Officer

Debbie Allen, Ph.D.
SVP, Business Development

Dirk Beeusaert
General Counsel




Thank you!