

Developing Highly Differentiated Antibody Therapeutics

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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 safety 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:** €162mm Sept 30, 2017 plus ~ €210mm net proceeds follow-on Dec 2017
- Blue chip investor base: more than 60% U.S. Shareholders
- 32.17 mio shares outstanding

Pipeline

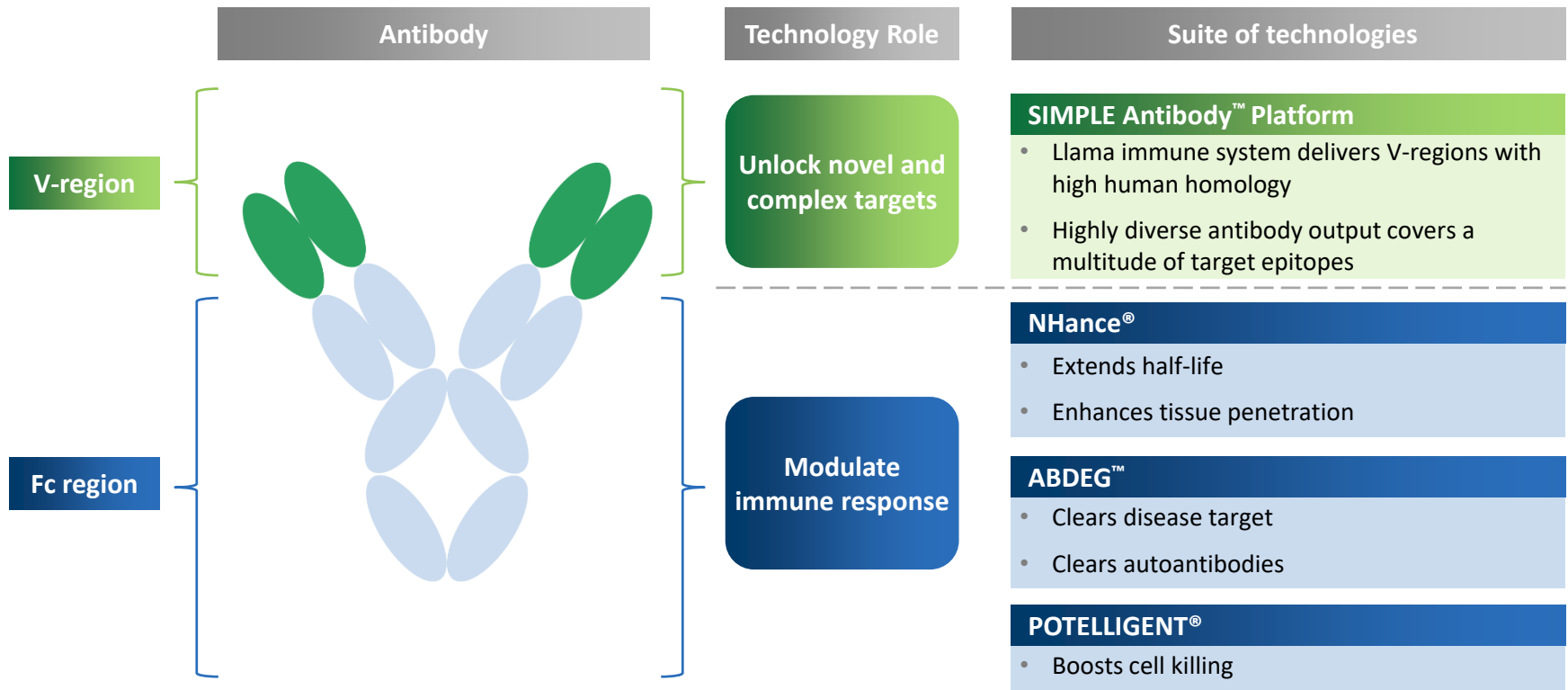
- ARGX-113 Ph2 MG: Topline data (Dec '17)
- ARGX-110 Ph1/2: Interim data AML and update CTCL (Dec '17)
- ARGX-113 Ph1 initiation in healthy volunteers with subcutaneous formulation (Oct '17)
- ARGX-113 Ph2 ITP: 50% recruited (Sept '17)
- ARGX-113 Ph2 initiation in PV (Sept '17)

Financing

- Upsized \$266 mm gross proceeds follow-on (Dec '17)
- Use of proceeds
 - Ph3 MG: launch and prepare production process for commercial production
 - Ph2 ITP & PV: clinical development and prepare for Ph3 (1 of these indications)
 - Ph1 subQ formulation: clinical development
 - Ph1/2 AML: clinical development




Maximizes value of our suite of technologies and capabilities





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



Product Candidate	Target	Indication	Preclinical	Phase 1	Phase 2	Phase 3	Next Milestone / Commentary
Wholly-Owned Product Candidates							
ARGX-113 (efgartigimod)	FcRn	Myasthenia Gravis	<div></div>				Positive Phase 2 topline results 
		Immune Thrombocytopenia	<div></div>				2H18: Phase 2 topline results
		Pemphigus Vulgaris	<div></div>				2H18: Phase 2 interim data
		Chronic Autoimmune Diseases	<div></div>			SubQ Formulation	2H18: Phase 1 interim data
ARGX-110 (cusatuzumab)	CD70	T-Cell Lymphoma	<div>Phase 1/2</div>				2H18: Phase 2 topline results CTCL
		Acute Myeloid Leukemia	<div>Phase 1/2</div>				2H18: Transition into Phase 2 in AML/MDS
ARGX-111	c-MET	Solid Tumors / Blood Cancer	<div></div>				Intend to partner
Partnered Product Candidates							
ARGX-109 (gerilimzumab)	 IL-6	Rheumatoid Arthritis	<div></div>				Eligible for up to €32.5mm in milestones, royalties & additional shares of Bird Rock stock
ARGX-112	 IL-22R	Skin Inflammation	<div></div>				Eligible for up to ~€100mm in milestones and tiered royalties
ARGX-115	 GARP	Cancer Immunotherapy	<div></div>				Received \$50mm so far; eligible for up to \$625mm milestones & tiered royalties
ARGX-116	 ApoC3	Dyslipidemia	<div></div>				Eligible for double-digit royalties and exclusive option to license the program

- We obtained the exclusive license option from **Broteio Pharma** for an antibody against a novel complement target
- We have an antibody discovery alliance with **Shire** focused on multiple rare disease targets

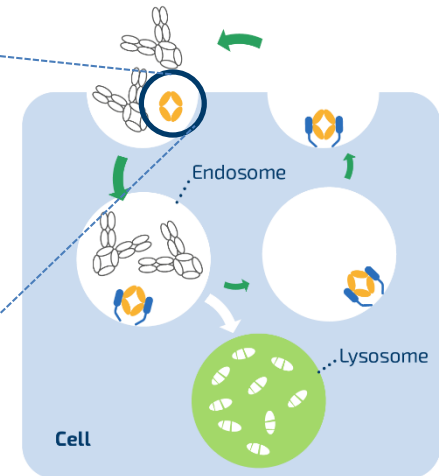
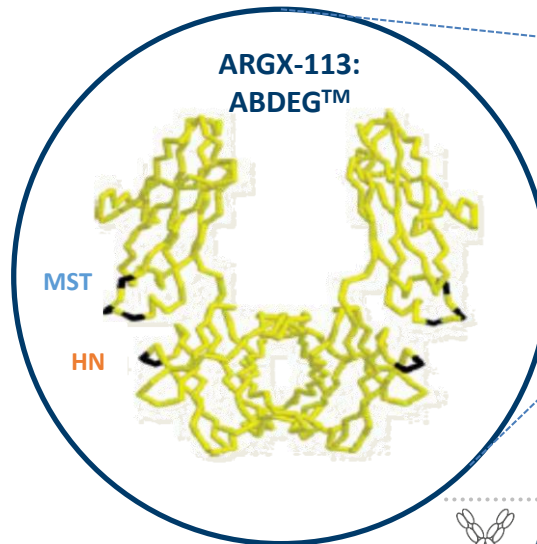
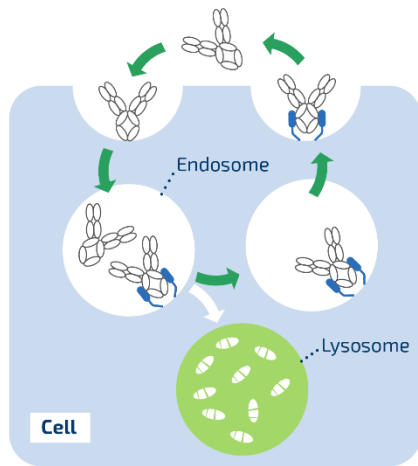
ARGX-113: A Pipeline-in-a-Product Opportunity

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

IgG antibodies recycle through FcRn⁽¹⁾...

...ARGX-113 potentially blocks FcRn...

...leading to IgG elimination



Antibody



ARGX-113



FcRn

- ARGX-113 is a human IgG1 Fc-fragment that utilizes ABDEG™ Fc engineering technology⁽²⁾⁽³⁾
- ARGX-113 does not expose the Fc tail and cannot engage Fcγ receptors
- 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

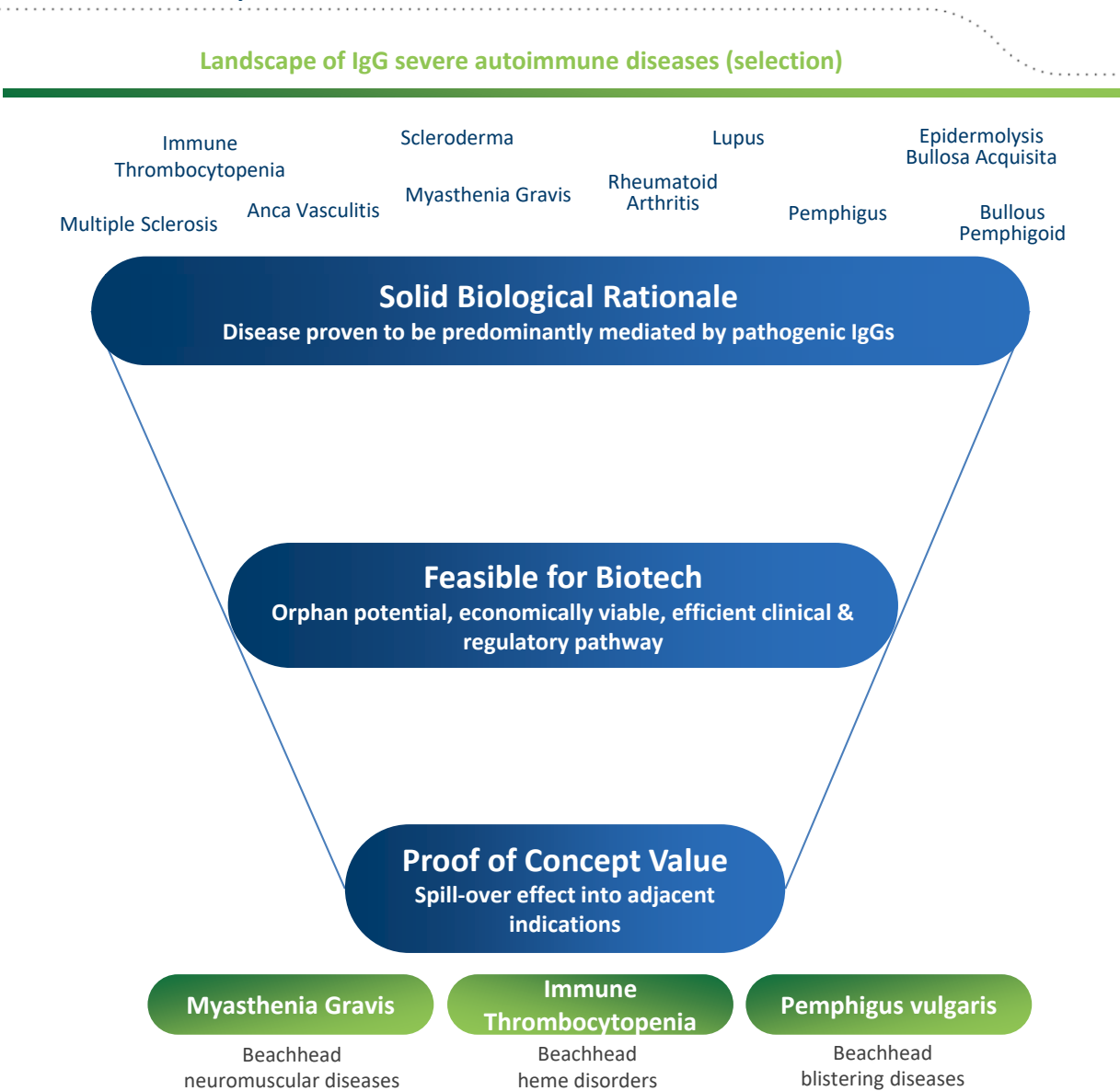
(1) Roopenian et al. 2007, Nat Rev Immunol.

(2) Vaccaro et al. 2005, Nat Biotech.

(3) argenx data

ARGX-113: Pipeline-In-Product Opportunity

Prioritizing IgG autoantibody mediated diseases



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), affecting all ages and both genders
- MG associated with muscle weakness; can be life threatening if respiratory muscles affected
- Symptoms include: Life-threatening choking; muscle dislocation; eyelid fatigue; pain; problems with vision, speech, mobility, fatigue

Limited current treatment options

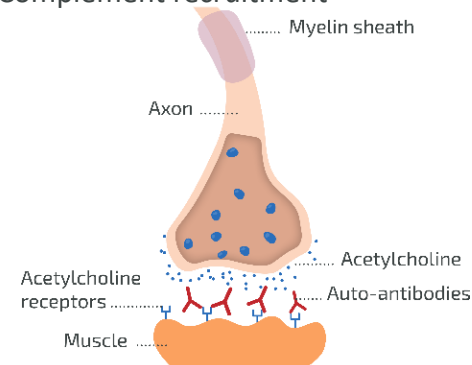
- Limited treatment options
 - Cholinesterase inhibitors
 - Corticosteroids
 - Immunosuppressants
 - IVIg, Plasmapheresis (exacerbations or rescue)
 - Soliris®
 - Thymectomy (minority of patients)
- Severe side effects of current treatment options: Injury, liver malignancy, osteopenia, osteoporosis, cataracts, depression, hypertension, hematologic suppression, headache, disfigurement, infection, thrombosis
- IVIg, Plasmapheresis and Soliris® place a heavy cost burden on healthcare systems (~\$79,000⁽³⁾, ~\$101,000⁽³⁾ and ~\$700,000⁽⁴⁾)



Myasthenia Gravis Cause

Autoantibodies (IgG type) destroy 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 Acquisition 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

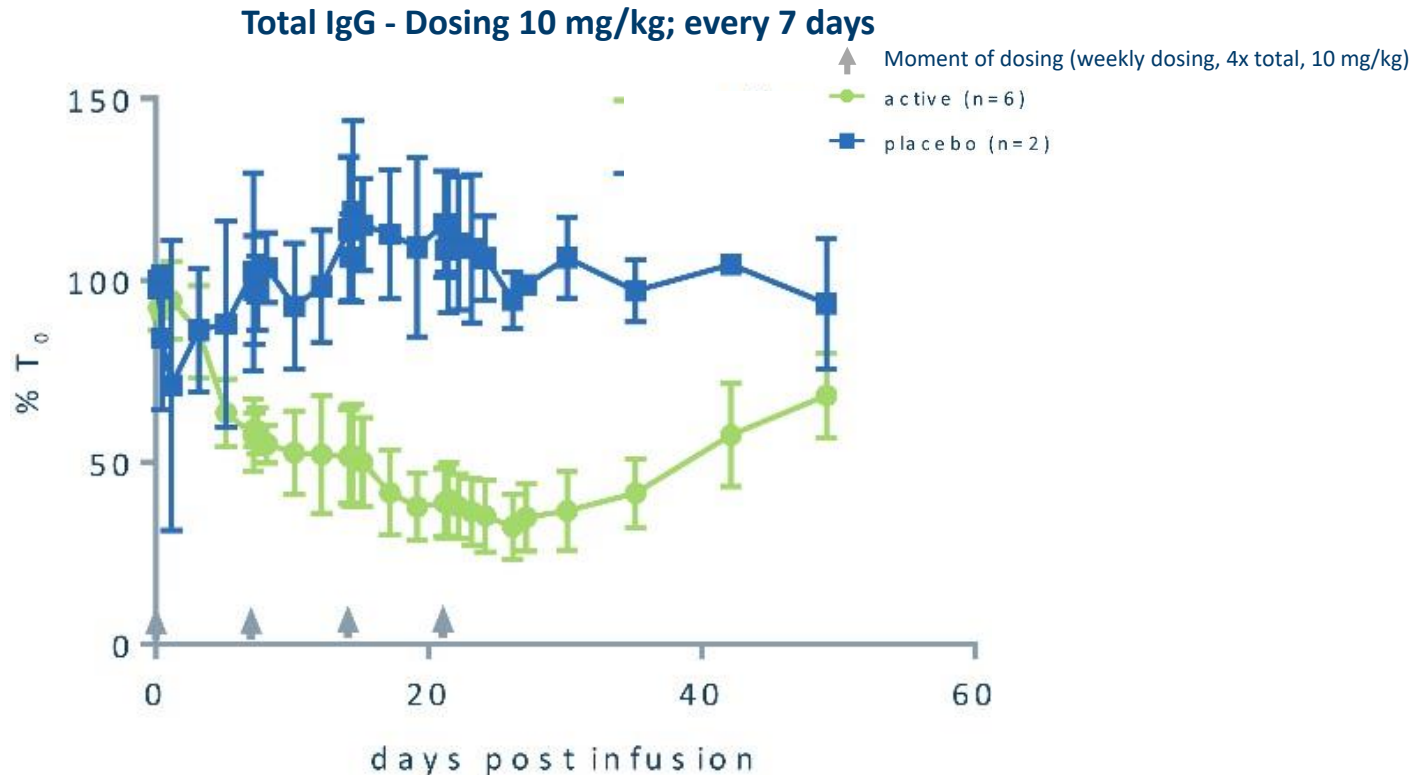
* Comparison between 3 cycles of Plasmapheresis/Immunoabsorption every 24h-48h and 5 cycles of IVIg every 24h

** 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

ARGX-113: Selective and Lasting IgG Reduction

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

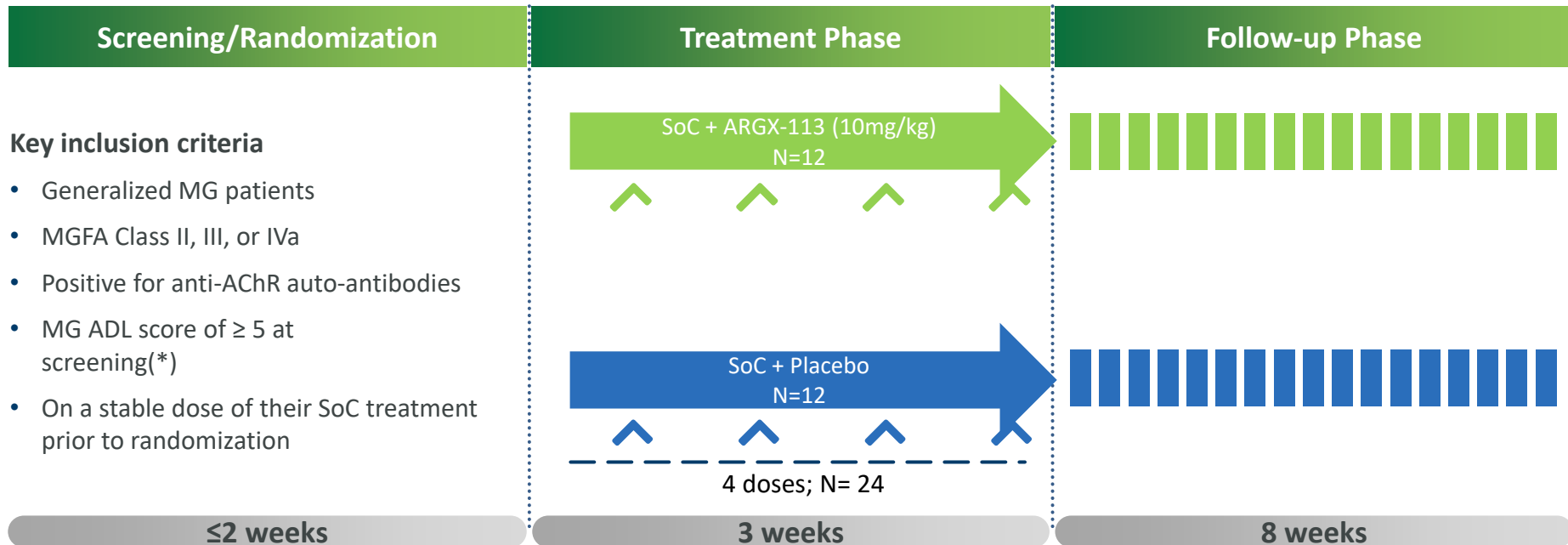


- Potent IgG reduction across isotypes (AChR autoantibodies are IgG1/3; MuSK autoantibodies are IgG4)
- Up to 85% total IgG reduction; single dose delivers 50% total IgG reduction
- After last dose, IgG levels remain reduced by 50% or more for ~3 weeks, return to baseline after > 1 month
- Comparable 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

Safety & tolerability

Secondary endpoints

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

MG Phase 2 Baseline Population And Disease Characteristics

	Placebo (N = 12)	ARGX-113 (N = 12)
Age (mean ± SD)	43.5 ± 19.3	55.3 ± 13.6
Sex (Number, %)		
• Male	4 (33.3%)	5 (41.7%)
• Female	8 (66.7%)	7 (58.3%)
Race		
• Asian	-	8.3%
• Black / African American	8.3%	-
• White	91.7%	91.7%
• Mixed / other	-	-
MGFA classification at screening*		
• Class I	-	-
• Class II	7 (58.4%)	6 (50.0%)
• Class III	4 (33.3%)	6 (50.0%)
• Class IV	1 (8.3%)	-
• Class V	-	-
Baseline QMG score (mean ± SD)	11.8 ± 5.4	14.5 ± 6.3
Baseline MG-ADL score (mean ± SD)	8.0 ± 2.2	8.0 ± 3.0
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 inhib. N (%)	11 (91.7%)	12 (100.0%)
• Corticosteroids N (%)	5 (41.7%)	8 (66.7%)
• Immunosuppressants N (%)	2 (16.7%)	9 (75.0%)



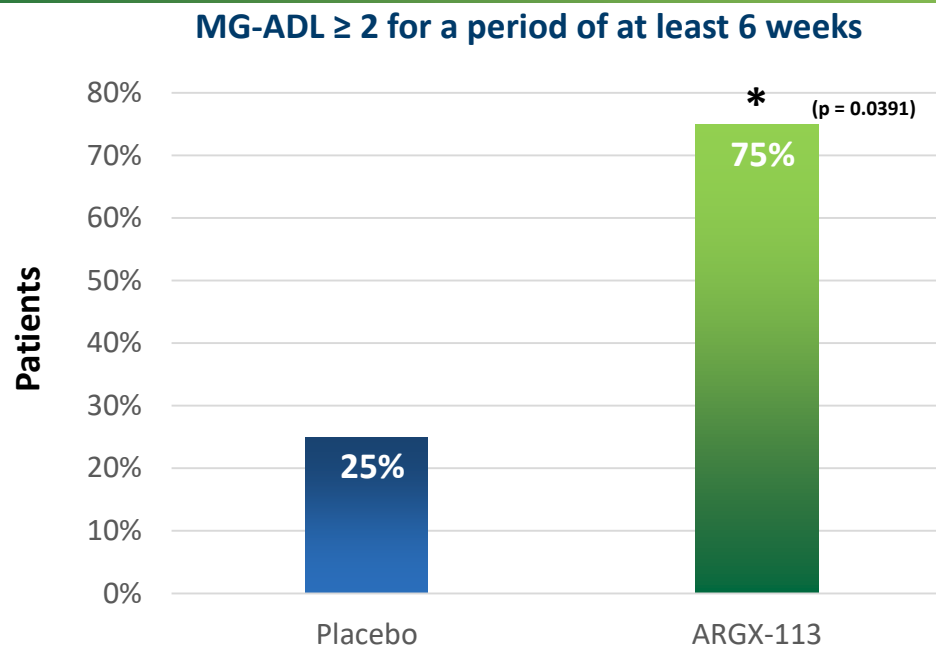
Favorable Safety And Tolerability Profile

Convenient 2h infusion enabling out-patient treatment

Treatment Emergent Adverse Events (TEAEs) Reported in ≥ 2 patients	Placebo (N = 12)	ARGX-113 (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%)	-
ARGX-113 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%)

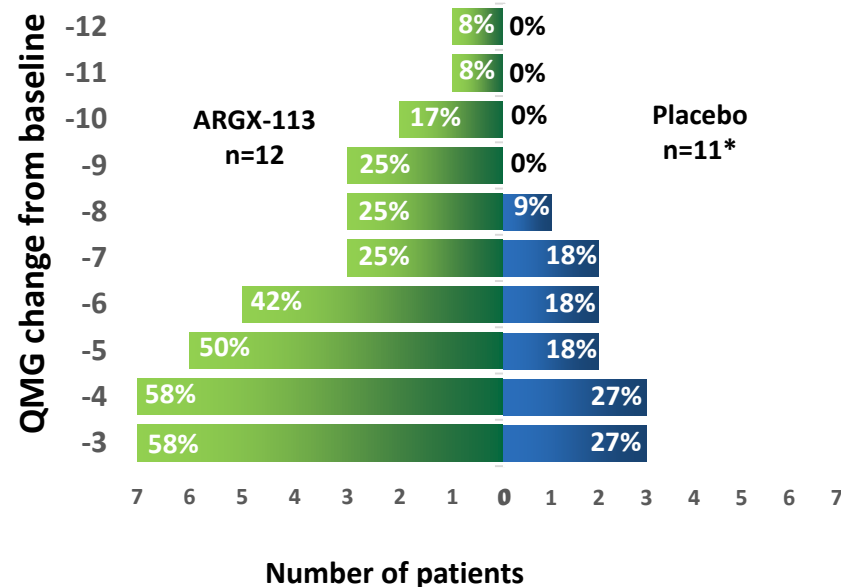
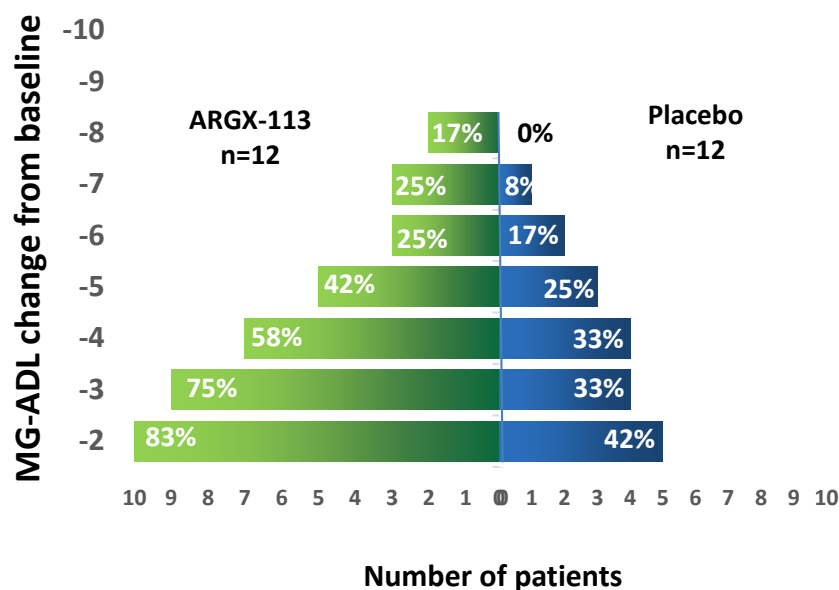
- ARGX-113 was well-tolerated in patients and confirmed findings from Phase 1 healthy volunteer trial
- The TEAEs profile was balanced between ARGX-113 and placebo
- TEAEs were mostly mild (grade 1) in severity. No severe AEs reported
- No deaths, Serious AEs or TEAEs leading to discontinuation of treatment were reported during the trial

75% Of ARGX-113 Treated Patients Achieved Lasting Response



- **83% of patients treated with ARGX-113** achieved a clinically meaningful response (MG-ADL ≥ 2)
- **75% of patients treated with ARGX-113** 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

ARGX-113 Group Showed Strong Clinical Improvement Over Placebo Group – Day 29 data (1 week post last dosing)



- Increasing differentiation observed between the ARGX-113 and placebo treatment group with increasing MG-ADL thresholds
- ARGX-113 treated patients showed rapid onset of disease improvement, with clear separation from placebo 1 week after the first infusion (data not shown)
- Disease improvement was found to correlate with reduction in pathogenic IgG levels
- ARGX-113 treatment resulted in a strong clinical improvement over placebo during the entire duration of the study as measured by all four predefined clinical efficacy scales

Transformational Data Set



Consistent and compelling safety & tolerability profile is a key differentiator in FcRn antagonist space



Fast, strong and sustained benefit; clinically meaningful and statistically significant



Strong correlation between IgG level reduction and disease improvement; validating focus on IgG-mediated diseases



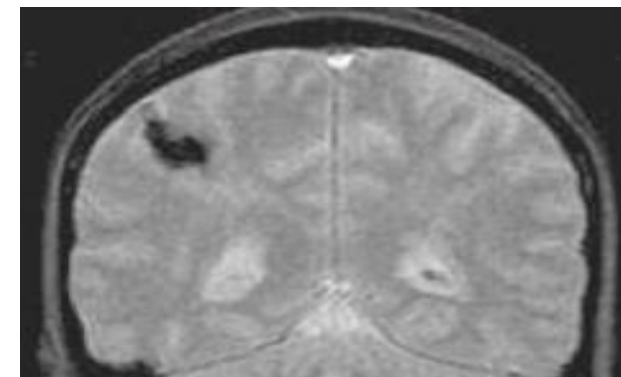
Phase 2 execution catapults ARGX-113 towards Phase 3

What is Immune Thrombocytopenia?

- Rare bleeding disease; estimated 72,000⁽¹⁾ patients in US, more frequent in females and patients over 60
- Symptoms range from mild bruising to severe bleeding
- Symptoms include: Fatigue, emotional strain, impact on work, fear of bleeding, impact on social activities, bruising

Limited current treatment options

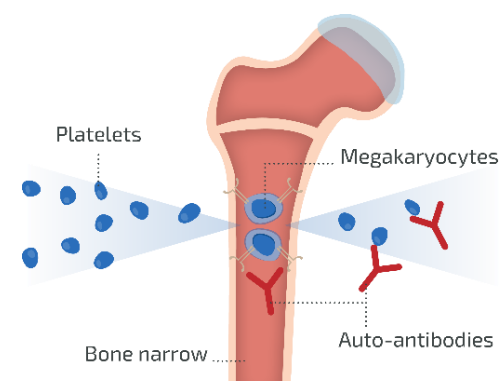
- Limited treatment options
 - Multiple iterations on corticosteroids & IVIg
 - Immunomodulatory agents
 - TPO mimetics & splenectomy
- Severe side effects from current treatments: Anaphylaxis, anorexia, backache, cancer, cataracts, depression, diabetes, fatal hemolysis, hepatitis, hypertension, infections, infusion-related reactions, leukoencephalopathy, nausea, osteoporosis, psychosis, sweating, neutropenia, thrombosis, vomiting, weakness
- Romiplostim and Eltrombopag, last-line therapies for ITP and have generated global revenues of \$584 million⁽²⁾ and \$635 million⁽³⁾ in 2016



Immune Thrombocytopenia Cause

Autoantibodies (IgG type) destroy blood platelets:

- Increased platelet removal
- Reduced platelet production

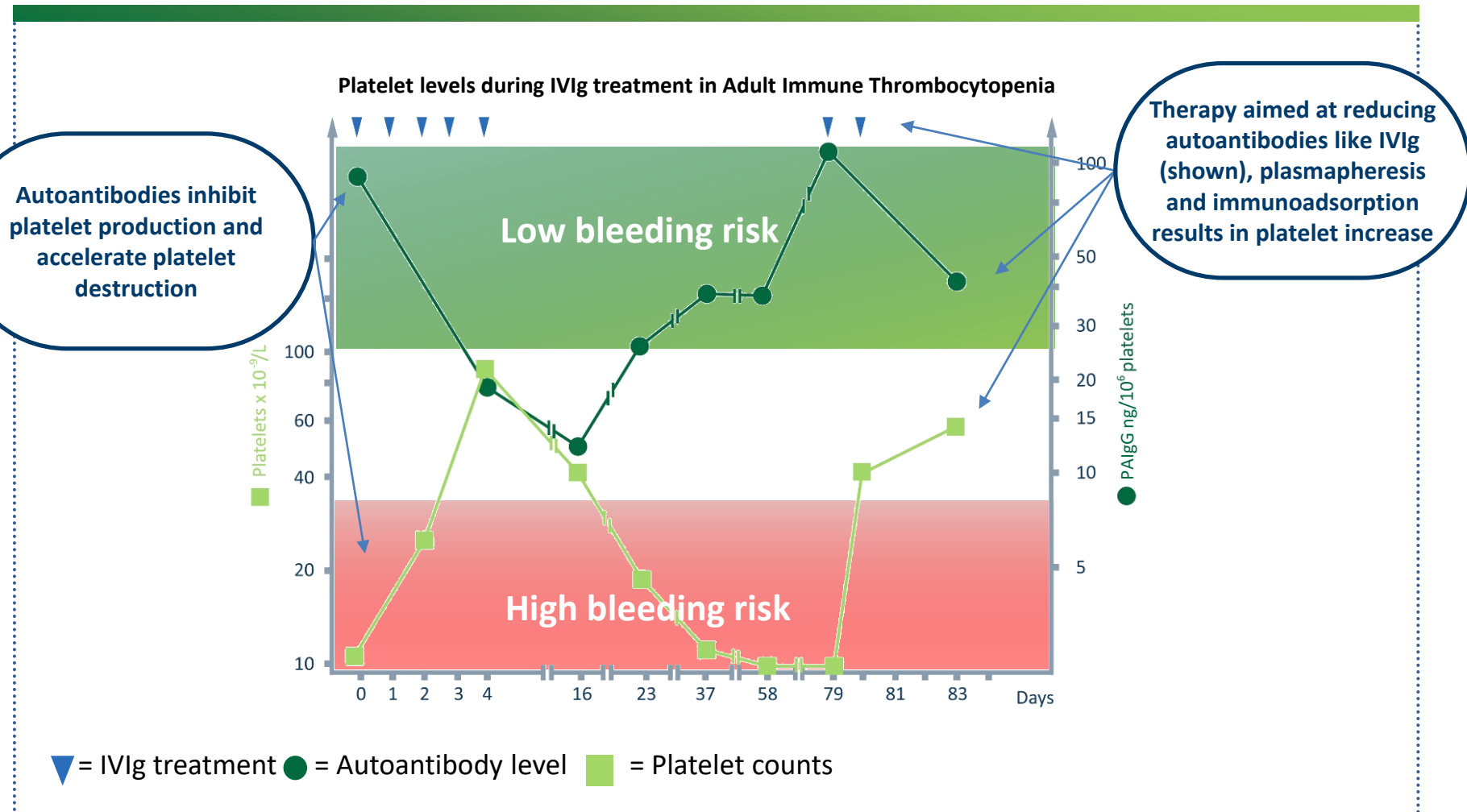


(1) Saleh et al. 2015, Curr Med Res Opin.; Terell et al. 2012, Am J Hematol.; Grace et al. 2012, Pediatr Blood Cancer.

(2) Amgen Inc. 2016, Form 10-K.

(3) Novartis Annual Report 2016

Autoantibody Levels (IgGs) Correlate With ITP Disease Score



What is Pemphigus Vulgaris?

- Chronic, severe – potentially life-threatening – auto-immune disease
- ~ 17,000 people treated (US)⁽¹⁾
- Mucosal and skin blisters leading to pain, difficult swallowing, skin infection
- Disease severity directly correlates to pathogenic IgG levels against desmoglein-1 (skin involvement) and desmoglein-3 (mucosal involvement)⁽²⁾
- Patients cycle through periods of remission and relapse for extended periods

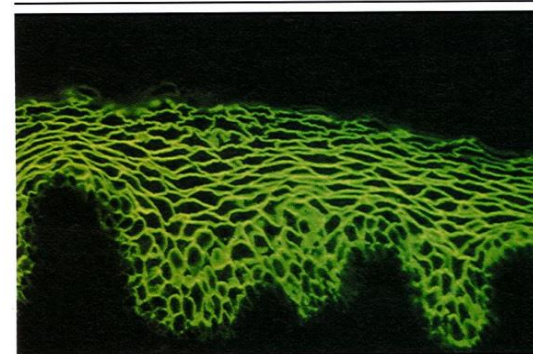
Limited current treatment options

- Current disease management comes with significant side effects and impacts QoL
 - High dose of corticosteroids and chronic immunosuppression (AZA, MFM)
 - Rituximab, IVIg, immunoadsorption and plasma exchange used for severe or refractory patients (10%), but not perceived as curative
- Treating physicians require new effective therapies with rapid onset of action that are safe
- Rituximab therapy shows slow onset of action, risk of developing serious adverse events and significant relapse rate ^{(2) (3) (4)}



Pemphigus Vulgaris Cause

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



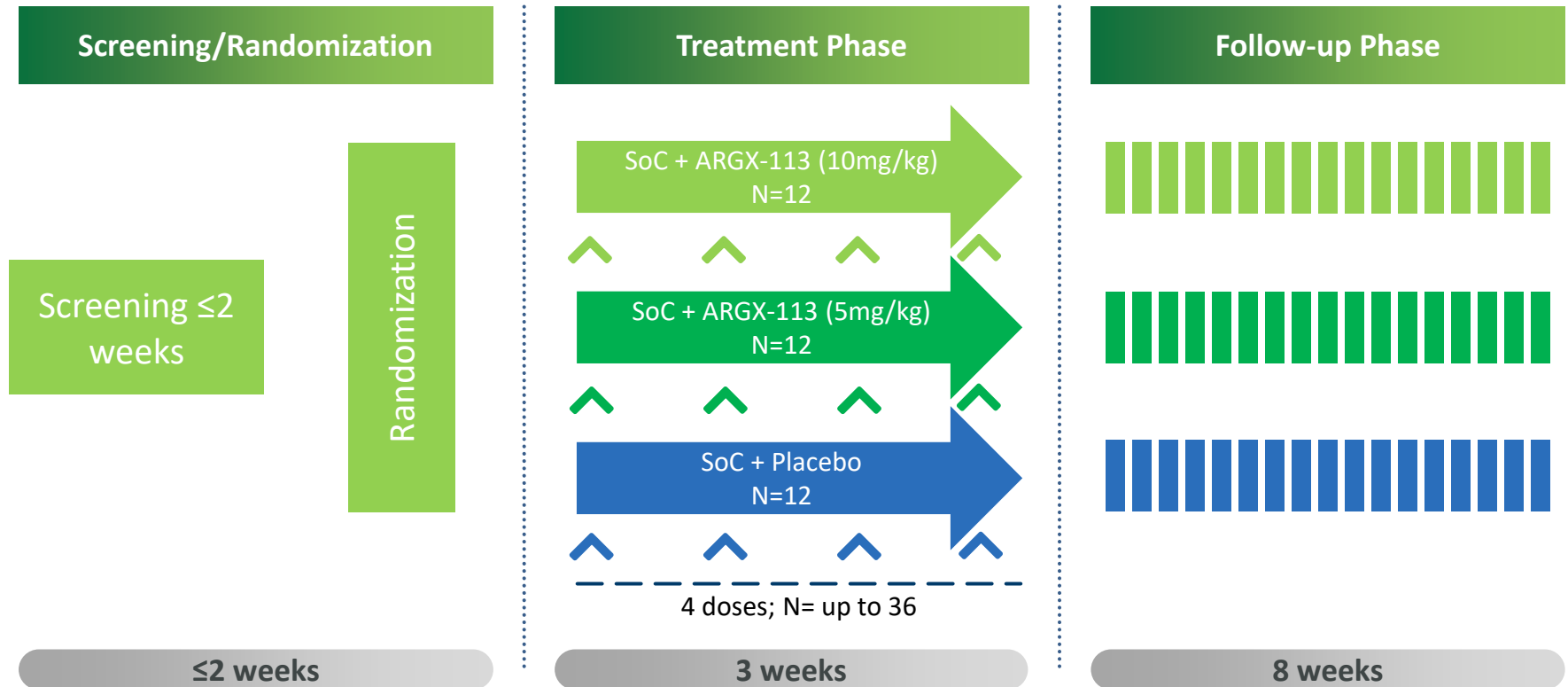
Auto-antibodies (predominantly IgG4 type) sterically hinder desmosomal adhesion and assembly – no complement involvement or immune effector activation⁽²⁾



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

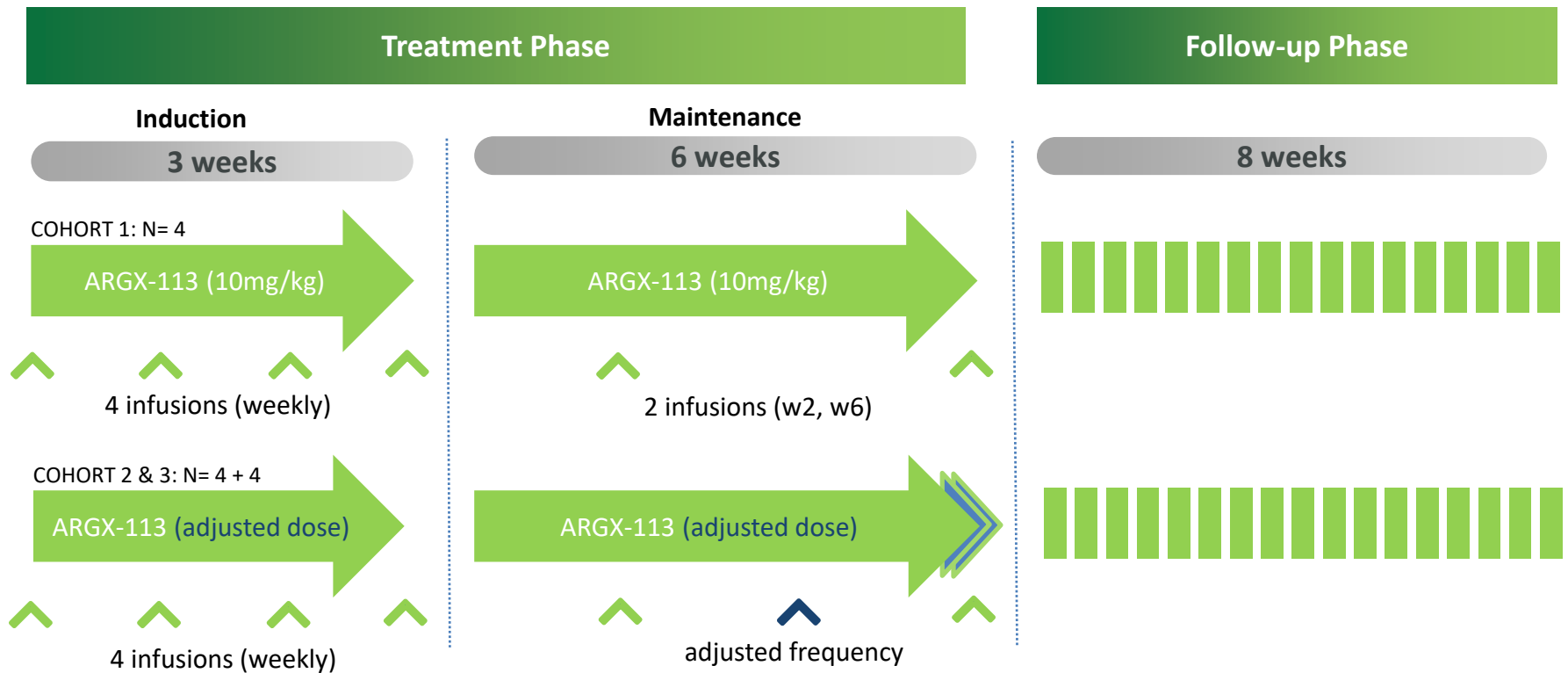
ARGX-113 in ITP: Phase 2 Trial Design

Study
50% recruited
Sept 2017



- Population: ITP patients with platelet levels $< 30 \times 10^9/L$
- Primary Objectives: Evaluate safety and tolerability
- Secondary Objectives: (i) Evaluation of efficacy based on platelet counts, use of rescue treatment & bleeding events
(ii) Assess pharmacokinetics (PK) and pharmacodynamics (PD) effect
(iii) Evaluate immunogenicity



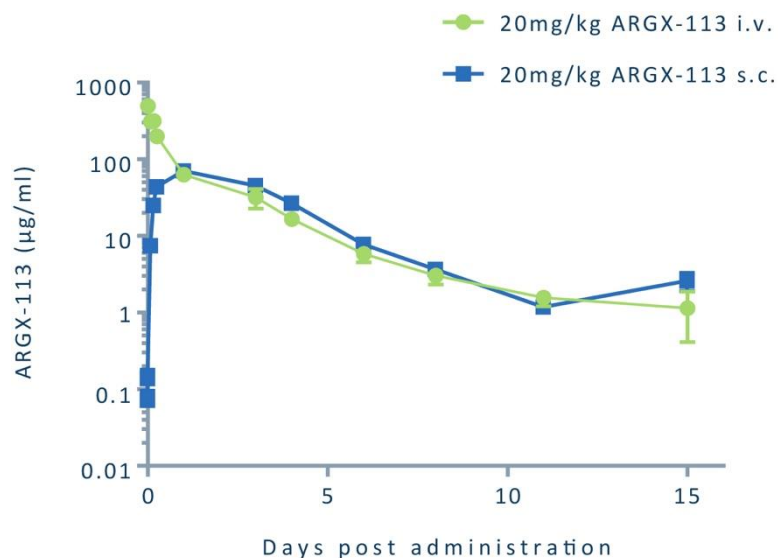


- Patients enrollment divided in 3 sequential cohorts
- IDMC recommendations for cohorts 2 & 3:
 - Change of dose (max dose of 25mg/kg)
 - Frequency of administration at maintenance (max 2 extra doses after each cohort)
 - Expansion of maintenance duration

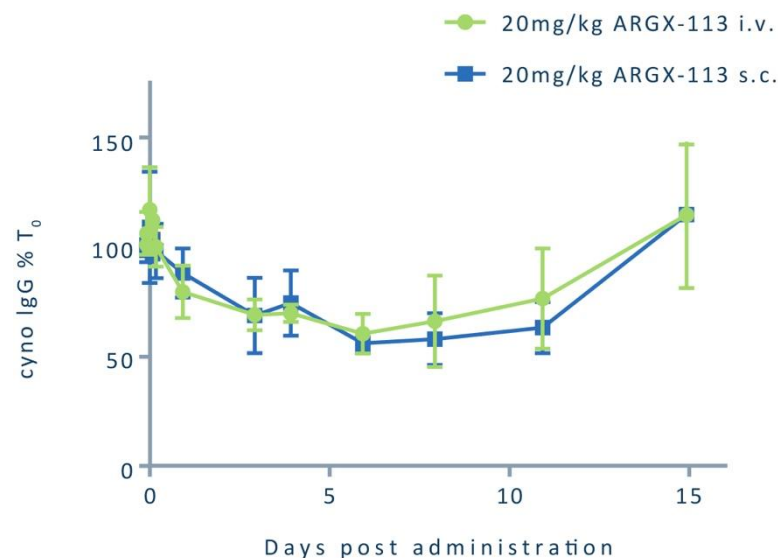
ARGX-113: Feasibility of SubQ Dosing

Exploring SubQ formulations for larger patient populations (chronic, ex-hospital)

PK single dose administration: IV vs SubQ (in cyno)



PD single dose administration: IV vs SubQ (in cyno)



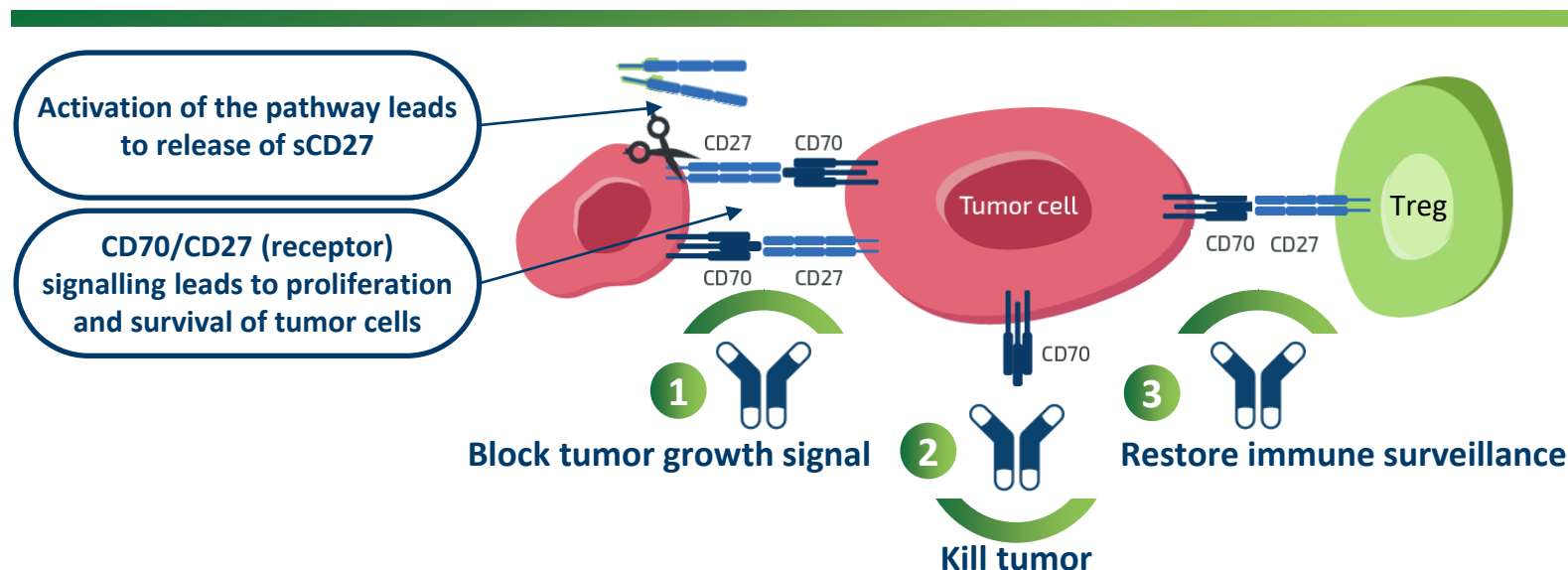
- Comparable PK and PD of IV versus SubQ dosing in preclinical studies demonstrated
 - Comparable half life
 - Favorable bio-availability of the compound in SubQ dosing (> 75%)
 - Comparable reduction of IgGs with single dose; up to 50%



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

What is Cutaneous T-Cell Lymphoma?

- Rare and incurable sub-type of T-cell lymphoma
- Prevalence (US & Canada): ~ 30,000 & Incidence (US): ~ 3,000⁽¹⁾
- Patients typically diagnosed in their 60s
- Mycosis fungoides (50%), Sézary syndrome most common types⁽²⁾
- Symptoms include: severe rash, itching, tumor, skin infections
- Skin infection often cause of death

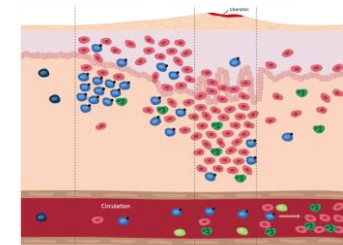
Limited current treatment options

- Initial treatment includes topical dermatology agents (corticosteroids, PUVA, e-beam therapy)
- Advanced stage patients treated with systemic oncology agents which are only moderately effective and not curative
 - Targretin bexarotene (oral) 1st line option – ease of administration
 - Istodax romidepsin (ORR: 34%, mDoR: 13-15 mos)⁽³⁾ 2nd line – complicated dosing and myelosuppression
 - Antifolates (methotrexate, pralatrexate), Campath, chemo (Doxil, CHOP, etc)
- Heavily pre-treated, elderly patients are unfit for aggressive chemotherapy or stem cell transplantation
- Significant unmet need for effective, tolerable, long-lasting CTCL treatments



Cutaneous T-Cell Lymphoma Cause

- Disease aetiology unknown
- Potentially caused by aberrant stimulation of CD4+ T-cells by Langerhans cells, specialized antigen presenting cells in skin
- Malignant T-cells become independent of stimulation by LCs and invade other tissues
- Sézary syndrome is a leukemic variant of CTCL



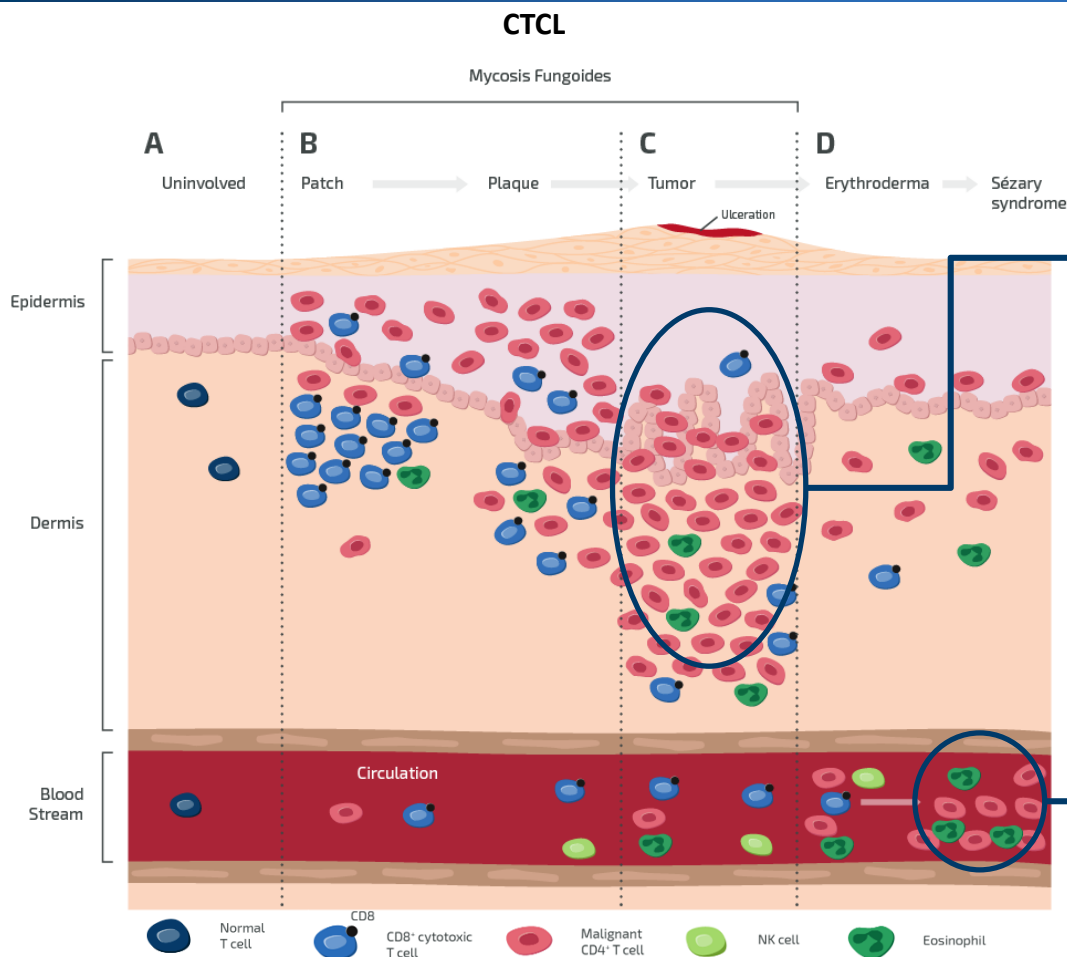
(1) Cutaneous Lymphoma Online Learning Center: <http://www.clfoundation.org/online-learning-center/disease/faq/who-gets-cutaneous-lymphoma-how-many-people-have-it>.

(2) Lymphoma Research Foundation: <http://www.lymphoma.org/site/pp.asp?c=bkLTkaOQLmK8E&b=6300151>

(3) <http://www.istodax.com/hcp/ctcl/study-design/efficacy>

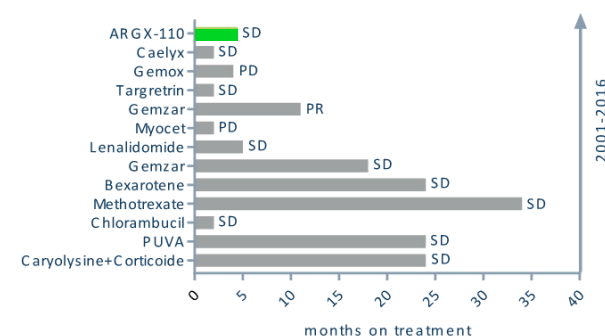
ARGX-110 In Cutaneous TCL

Phase 1-2: Typical patients are elderly and failing multiple lines of previous treatment



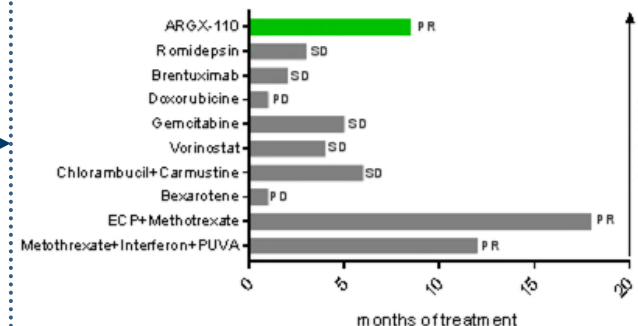
Example Mycosis fungoides (MF) patient

Treatment & best response



Example Sézary Syndrome (SS) patient

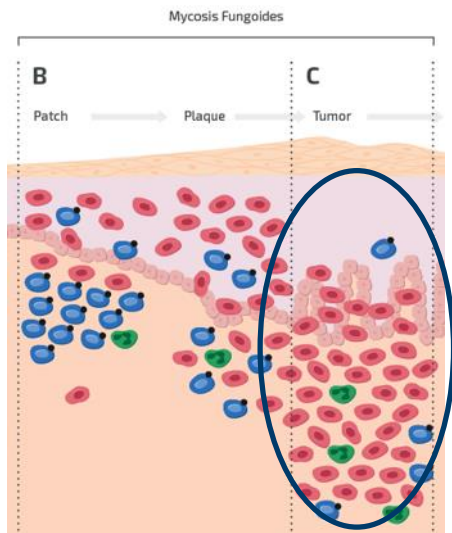
Treatment & best response



ARGX-110: Effect On Malignant Cells In Skin

Patient example 1: Cutaneous TCL – mycosis fungoides (MF)

Typical Mycosis fungoides (MF) patient



Patient	67 year old male CTCL-MF, diagnosed 2001
Tumor	Skin T4, Nx, M0, B0 (Stage IIIA)
Doses	6

Decrease of CD4+ malignant T-cells

Depletion of CD70+ malignant T-cells

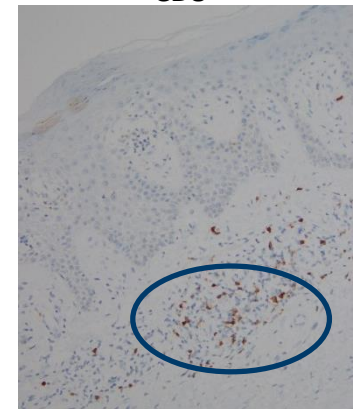
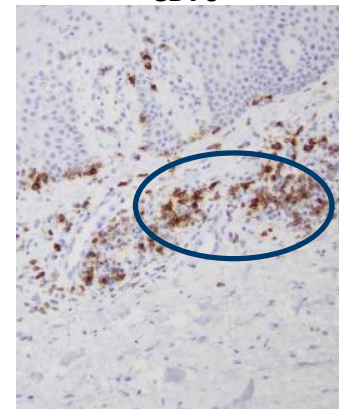
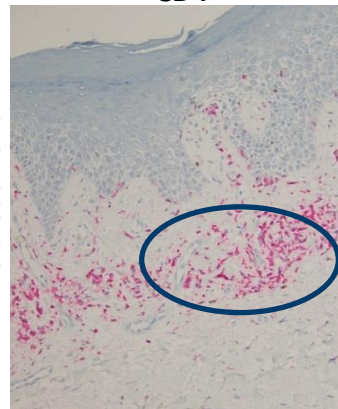
Infiltration of CD8+ T-cells

CD4⁺

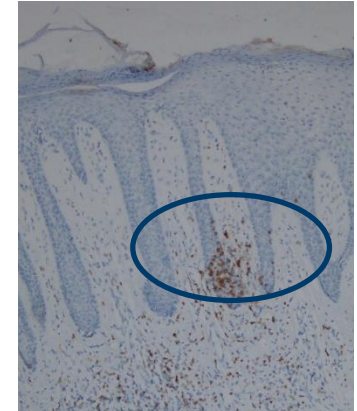
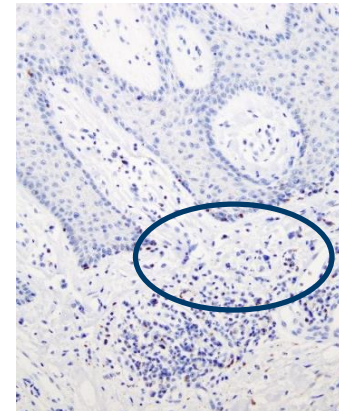
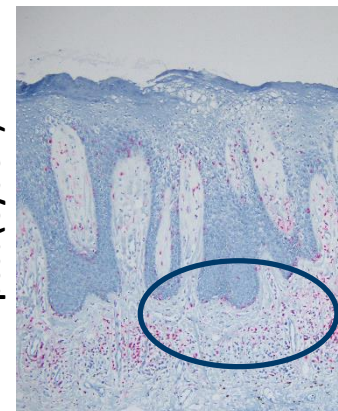
CD70

CD8⁺

Pre-treatment



post (Cycle 2)



ARGX-110: Improved mSWAT & Skin Lesions

Patient example 2: Cutaneous TCL – mycosis fungoides (MF)

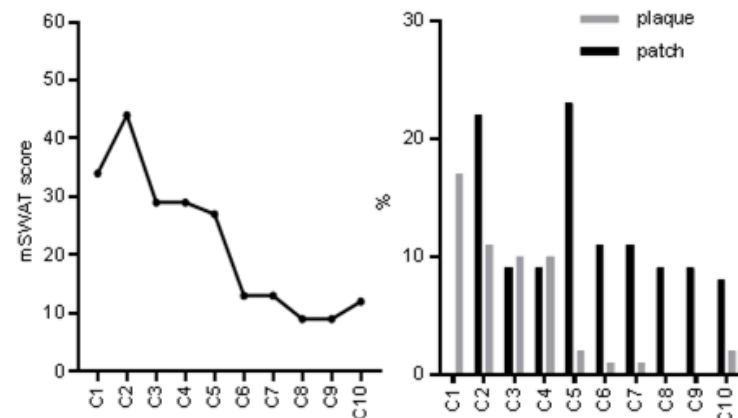


- 79 year old female with CTCL-MF, diagnosed June 2007
- Tumor: Skin T2, NO, MO, BO (stage IB)
- Doses: 16 (1 mg/kg q3w)

Pre treatment



At cycle 6



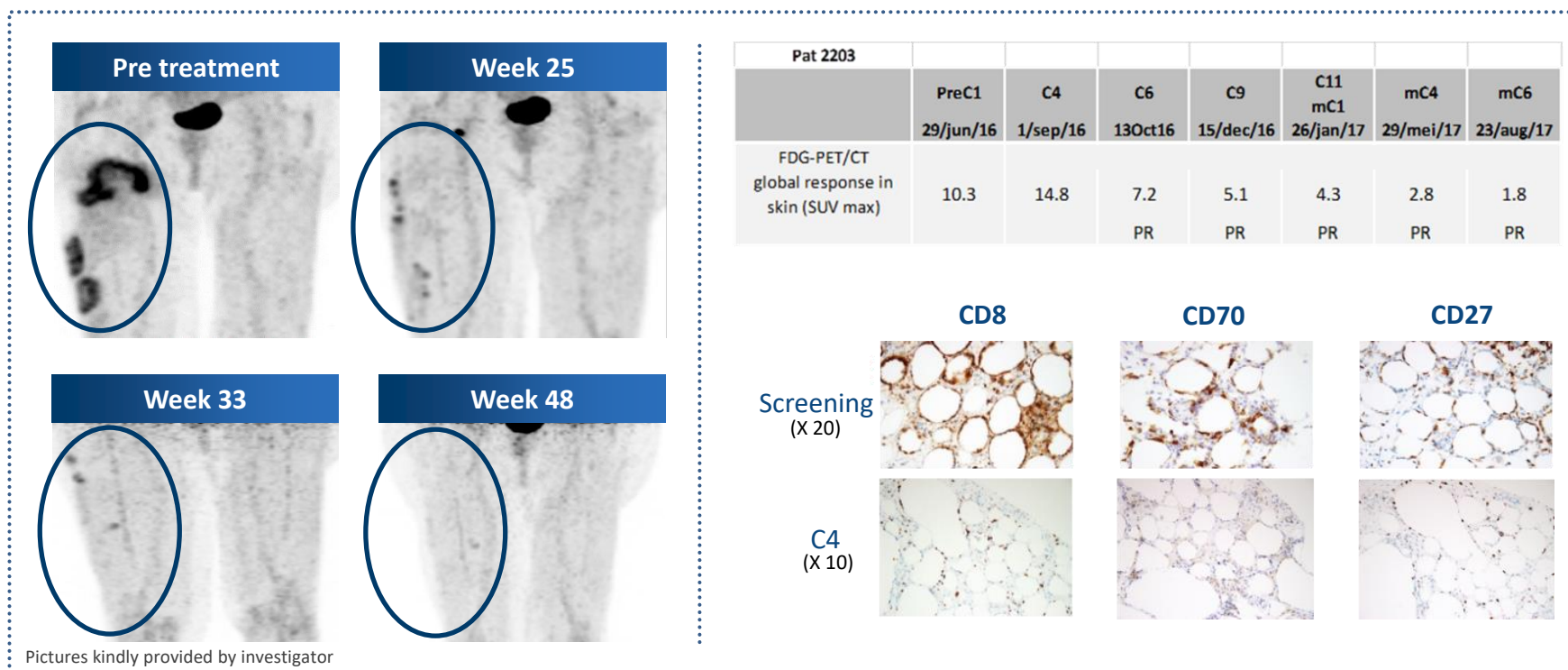
- 60% reduction of mSWAT score constitutes a partial response (PR)
- Decrease in surface area of cutaneous tumor lesions
- Lesions improve from plaques to patches
- Some lesions completely resolved

ARGX-110 Induces Complete Response

Update on panniculitis patient



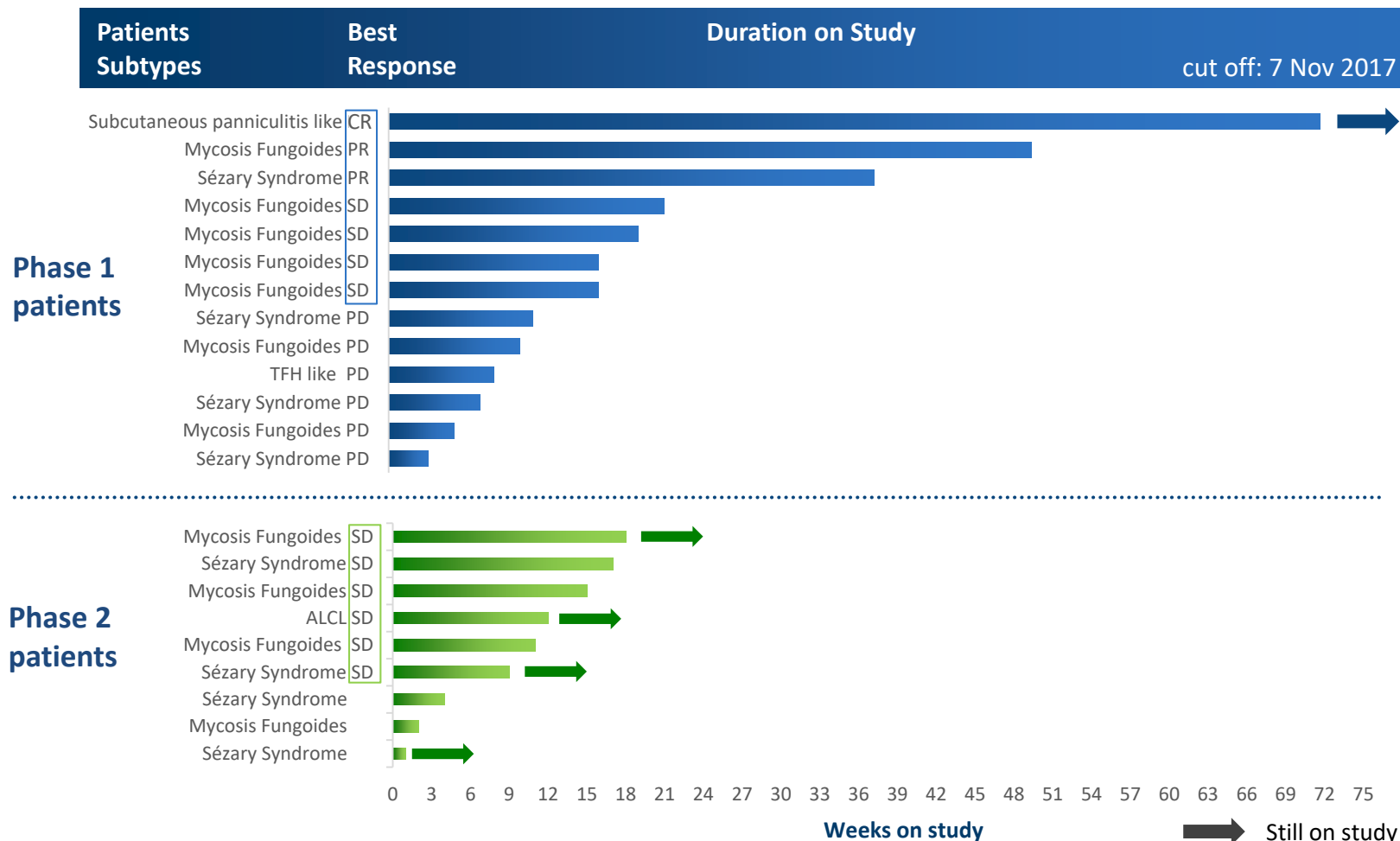
- 84 year old female, diagnosed June 2015
- Tumor: Skin T3, nodal NO, visceral MO, blood BO
- Doses: 10 (1 mg/kg q3w) + 8 (5 mg/kg q6w)



- Partial response after 6 doses (dose 1 mg/kg) in maintenance (5 mg/kg /6 weeks) since January 2017
- Complete response after 17 doses (dose 5mg/kg)
- The patient is still on a maintenance dose of 5 mg/kg q6wk

Disease Control In 59% (13/22) Of RR-CTCL patients

Duration on study



- Encouraging signs of clinical activity
- 5 patients still on study at 5 mg/kg

What is Acute Myeloid Leukemia?

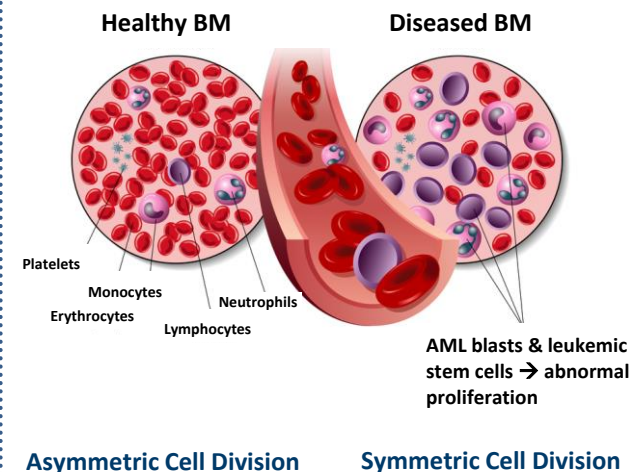
- Rare hematologic cancer characterized by **excessive proliferation of myeloid stem cells** and their **failure to properly differentiate into mature white blood cells**
- Symptoms include: weight loss, fatigue, fever, night sweats, loss of appetite, shortness of breath, easy bruising, infections, bleeding
- Disease progresses very rapidly and is fatal if left untreated
- ~22,000⁽¹⁾ new cases per year in the U.S. — **2nd most common leukemia subtype** in adults
- Generally a **disease of the elderly** — 60% of diagnosed patients are older than 60

Limited current treatment options

- **Elderly, frail patients** are typically unfit for high dose chemotherapy — receive **palliative treatment with hypomethylating agents**
 - Median survival of 7 – 10 months
 - 5 year survival rate of ~6%⁽²⁾ for patients over 65
- Younger patients (<45yr) typically get aggressive chemotherapy (“7+3” regimen) to induce remission followed by stem cell transplant
 - 5 year survival rate of ~57%⁽²⁾ for patients under 45
- **Significant need for safer and more effective treatment options**



Effects of AML on Bone Marrow

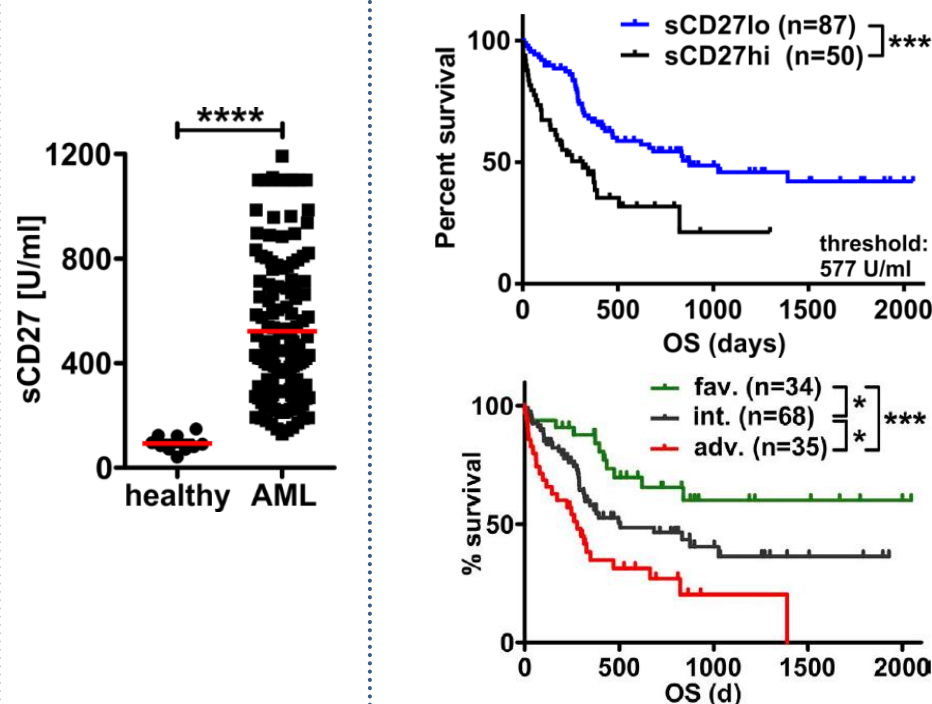


(1) American Cancer Society: <http://www.cancer.org/cancer/leukemia-acutemyeloidaml/detailedguide/leukemia-acute-myeloid-myelogenous-key-statistics> .
(2) National Cancer Institute: Howlader et al. (eds). SEER Cancer Statistics Review, 1975-2013, National Cancer Institute. Bethesda, MD, http://seer.cancer.gov/csr/1975_2013/,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

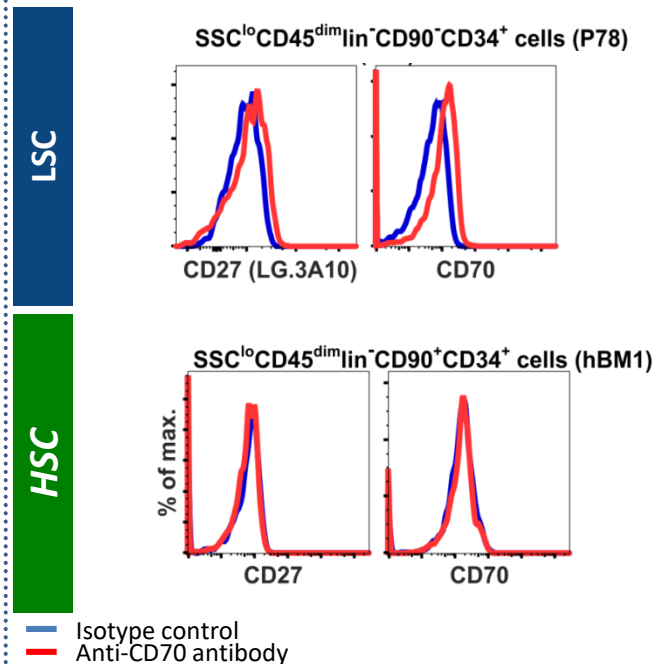
CD70 Provides Unifying Rationale Across Risk & Age Classes In AML

Potential to selectively target leukemic stem cells in AML patients

Elevated sCD27 serum levels correlate with poor prognosis



CD70 is a selective LSC marker

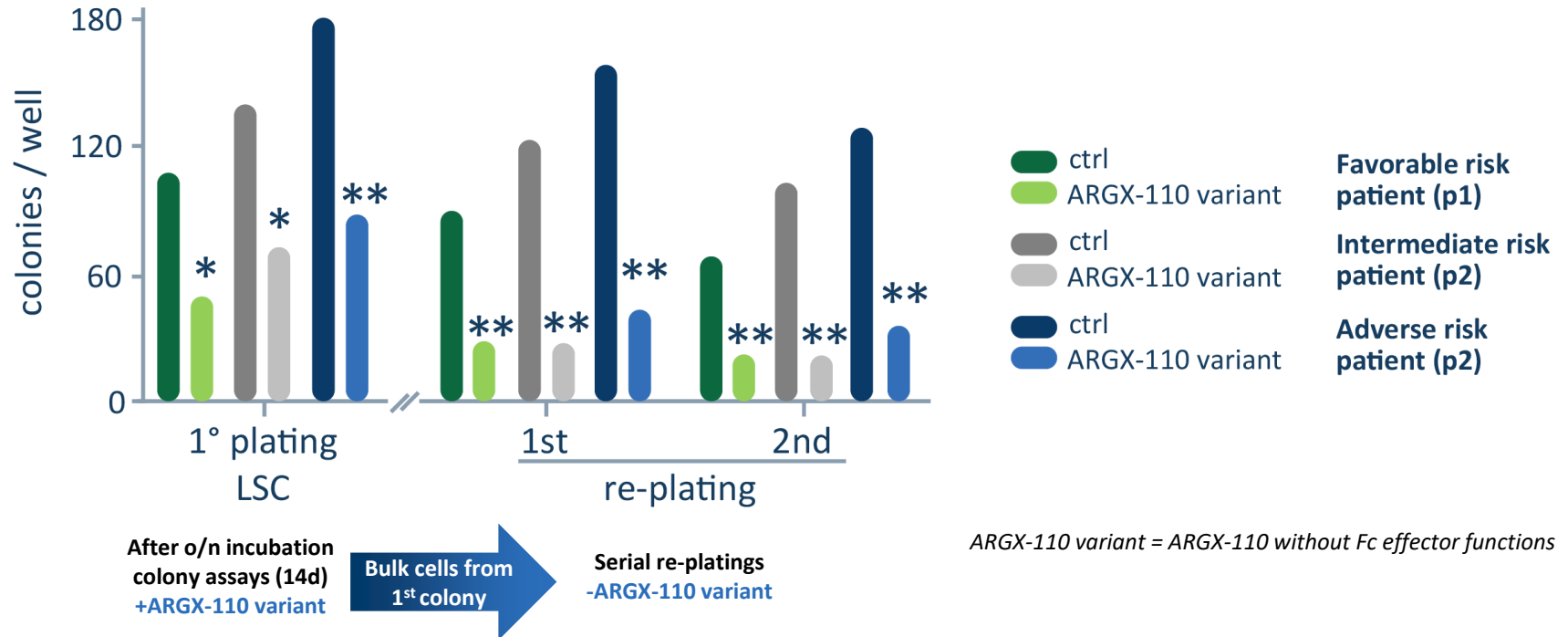


Legend: adv., adverse; CI, confidence interval; fav., favorable; int., intermediate; OS, overall survival. Statistics: left: one-way ANOVA; middle: log-rank test. *, P < 0.05; **, P < 0.01; ***, P < 0.001.

- 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)

ARGX-110: Inhibits LSC Proliferation In Lasting Fashion

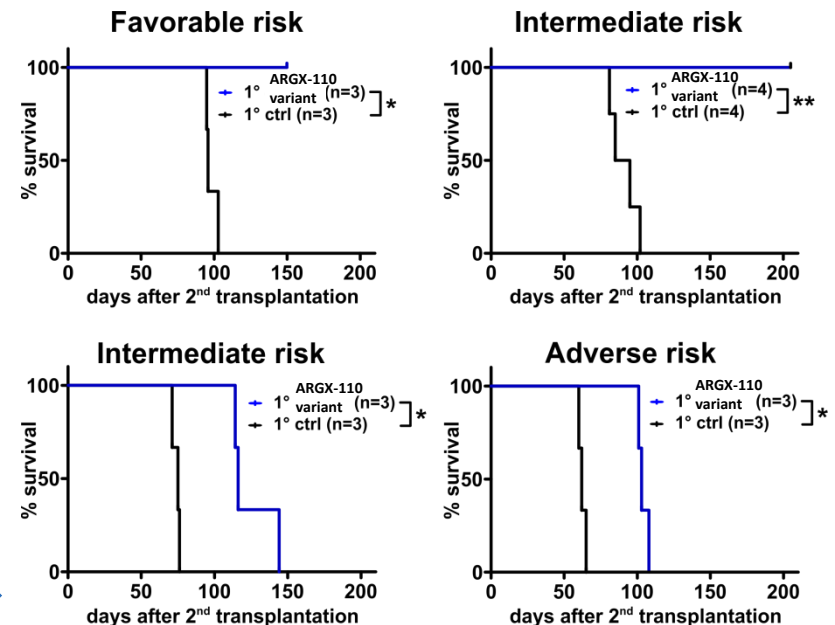
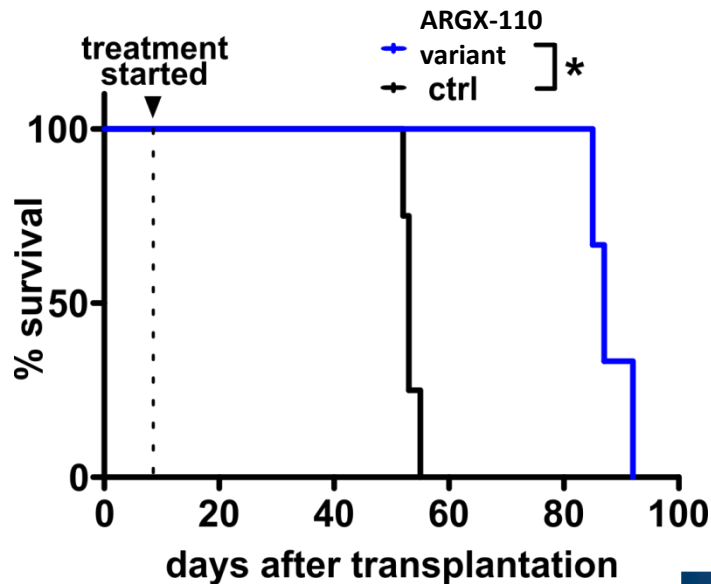
Long-term effects *ex vivo*



- 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

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)

Phase 1 – Dose escalation

Safety and tolerability

We are here

Vidaza = 75 mg/m² (standard of care)

10 mg/kg

N = 3+3

3 mg/kg

N = 3+3

1 mg/kg

N = 3+3

N = up to 18

Phase 2 – Proof-of-Concept

Efficacy

selected ARGX-110 dose

N = 15

selected ARGX-110 dose

N = 9 – (3-6 from Ph 1)

N = up to 21

- Hypomethylation agents such as Azacitidine increase CD70 expression¹
- 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

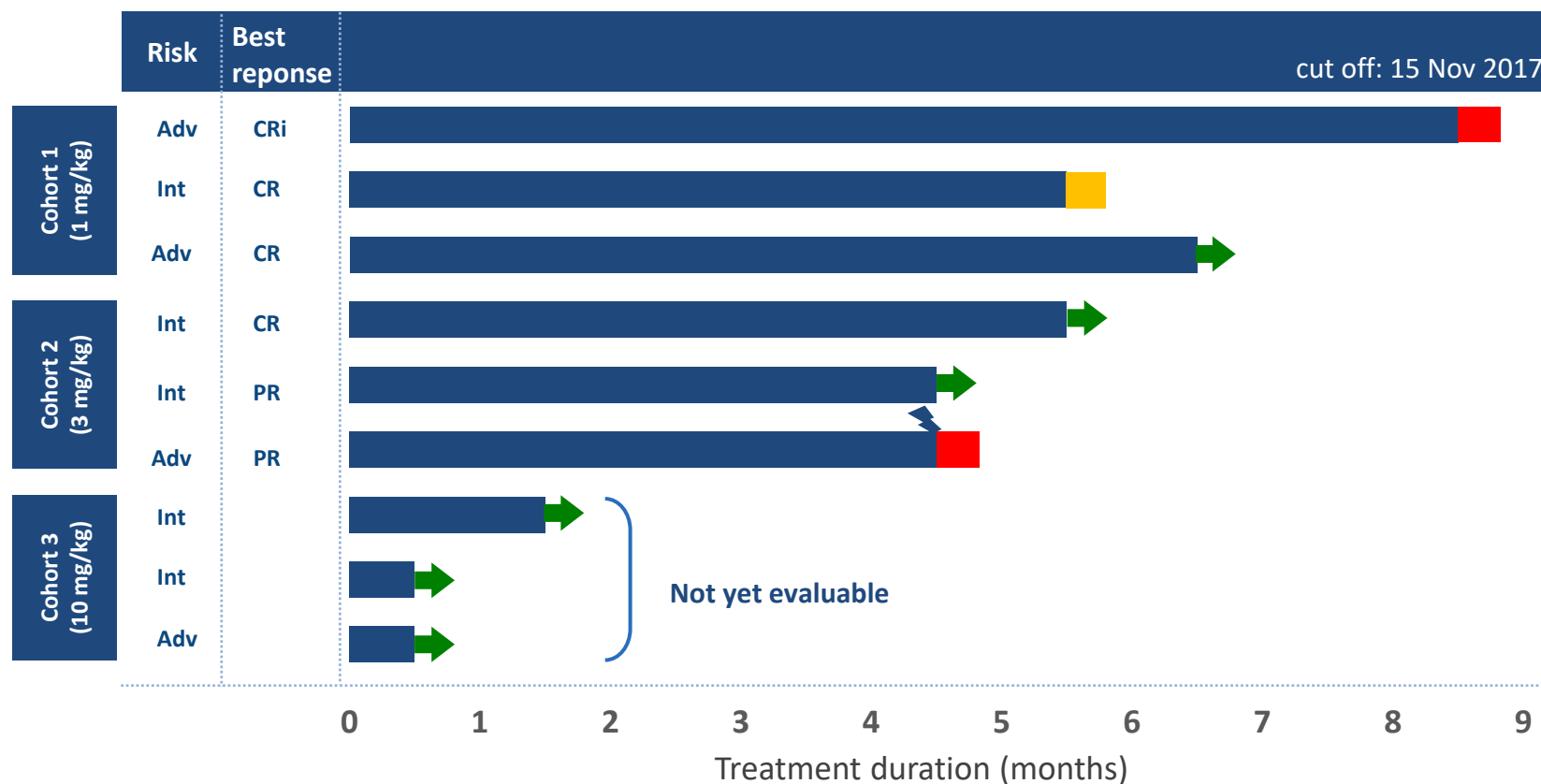
9 newly diagnosed AML patients

Baseline characteristics (N=9)	ARGX-110 + Azacitidine			Total
	1 mg/kg	3 mg/kg	10 mg/kg	
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	



Response in 6/6 Evaluable Newly Diagnosed AML Patients

ARGX-110/Aza treatment



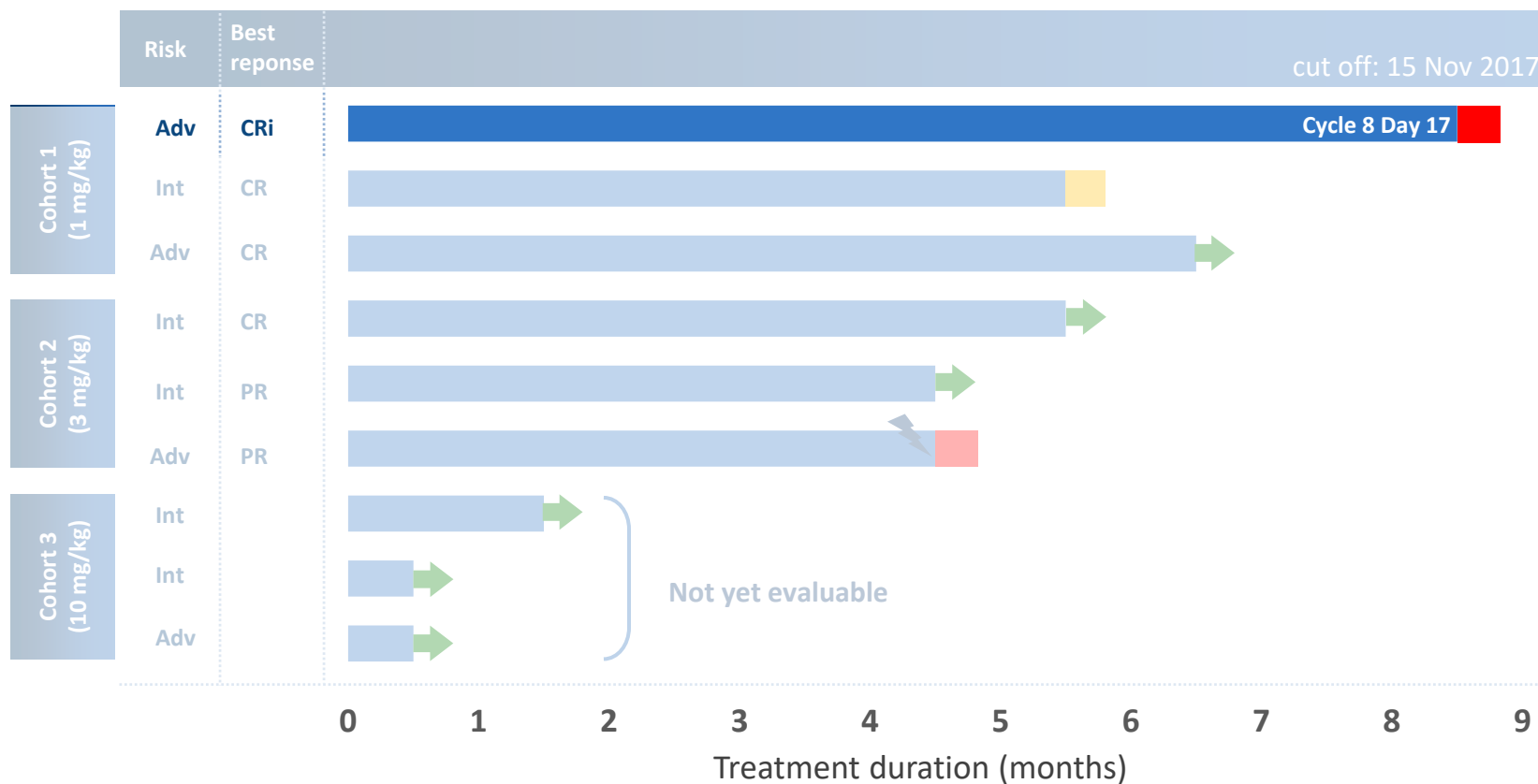
- So far, all patients responded (3 CR, 1 CRi, 2 PR)
- 1 patient reached CR and bridged to allogeneic stem cell transplant after 5 cycles
- 6/9 patients are currently still on treatment

- Study ended
- Patient successfully transplanted
- ⚡ Adverse event leading to discontinuation
- ➡ Ongoing study

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

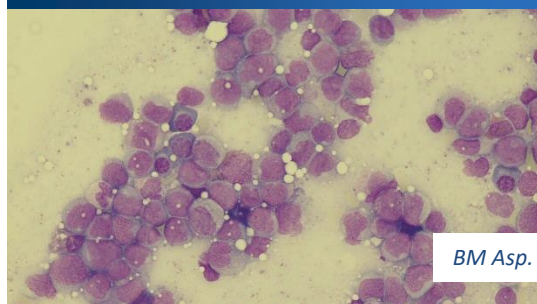


- 80 year old female
- Therapy-related AML, M4; BM ~65% blasts
- Molecular genetics: FLT3-ITD; DNMT3A mut; RUNX1 mut; WT1 mut; cytogenetics: normal

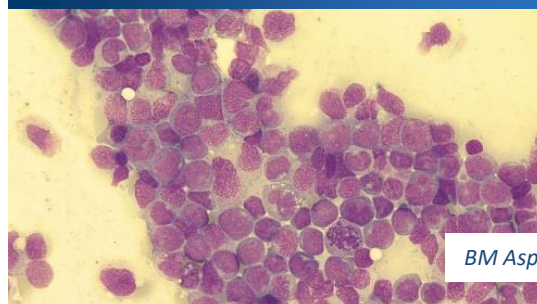


Case 1: Complete remission with incomplete hematological recovery

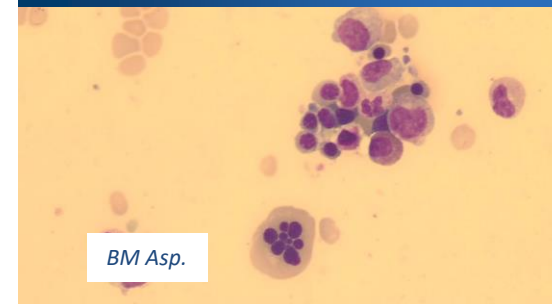
Screening



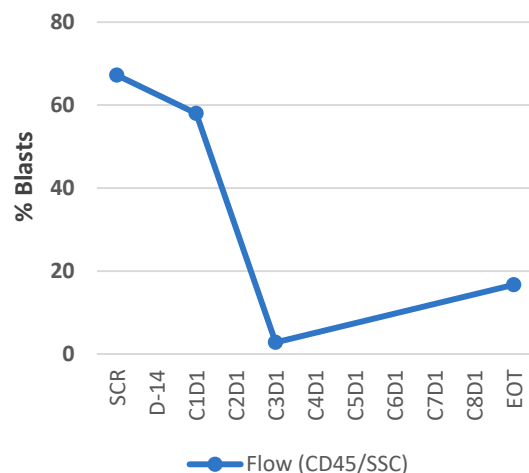
Leukemic blast persistence – (C1D1)



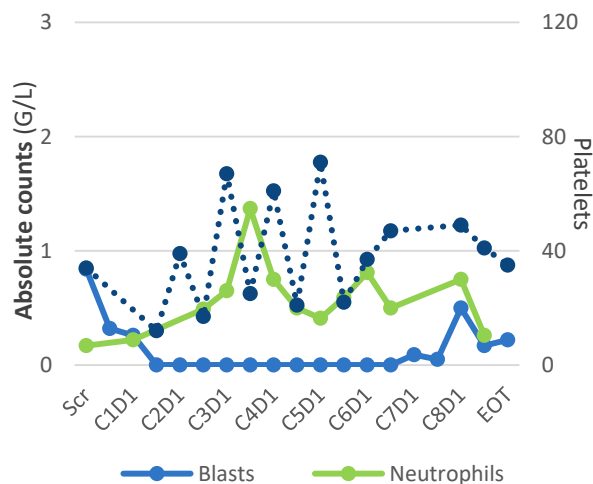
Leukemic clearance – (EOT)



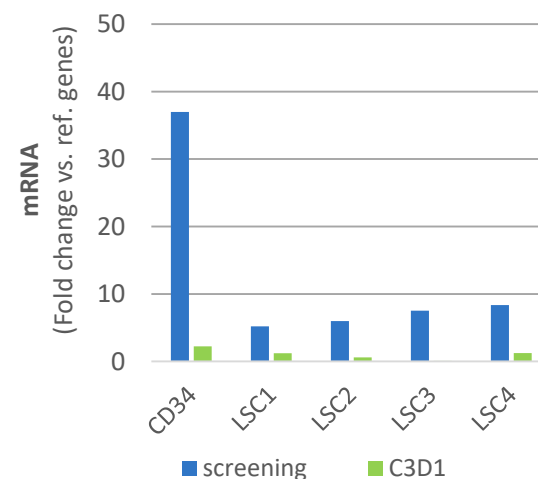
Bone marrow:
% Blasts, flow cytometry



Blood analysis:
Absolute counts (G/L)



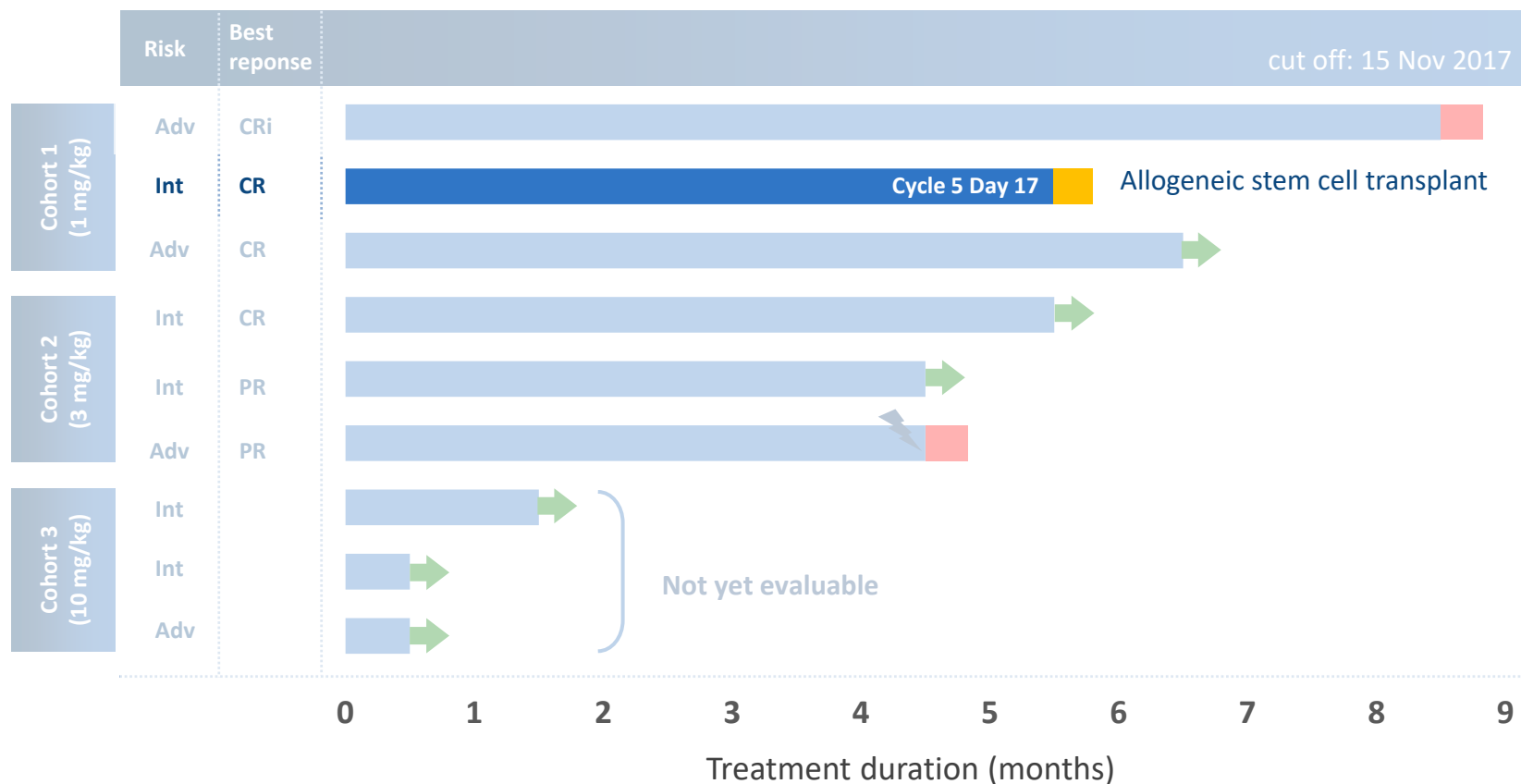
ARGX-110/Aza reduces
experimental LSC gene signature



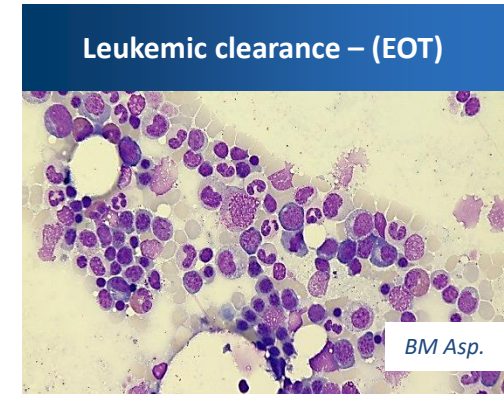
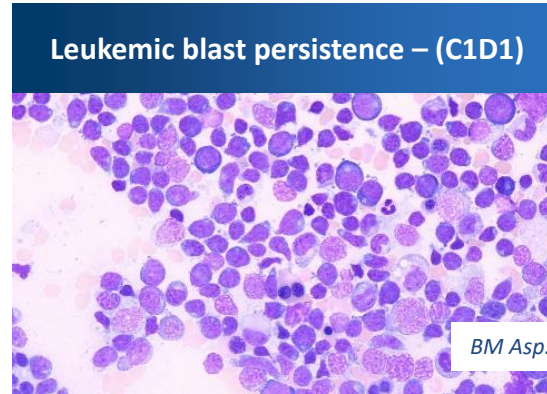
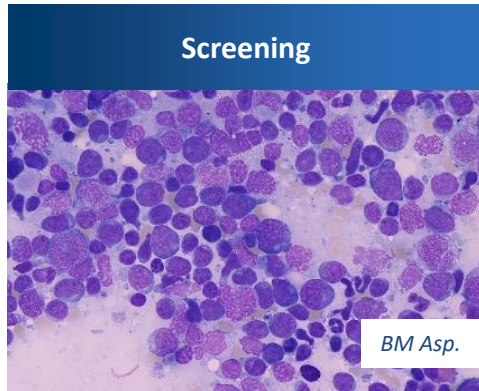
Case 2: Patient Cohort 1 – 1 mg/kg – 5 Cycles on Study



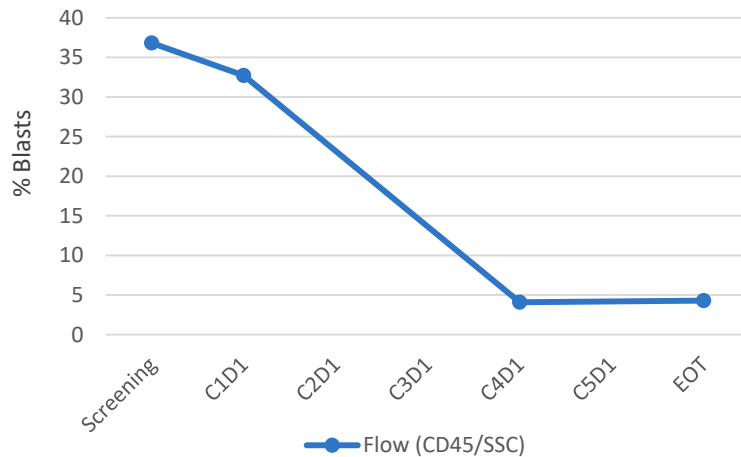
- 75 year old male
- AML with myelodysplasia-related changes, M1/M2; BM ~40% blasts
- Molecular genetics: U2AF1mut; DNMT3Amut; cytogenetics: normal



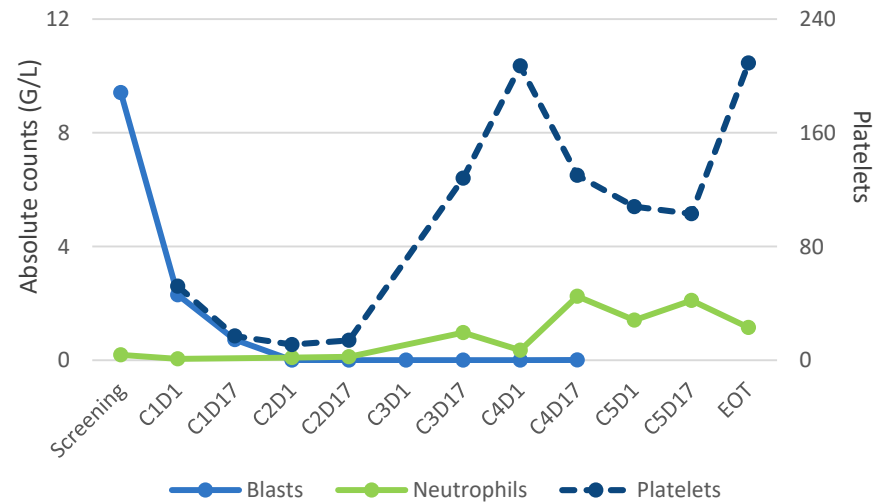
Case 2: ARGX-110/Aza Induces Complete Remission & Bridges to Transplant



Bone marrow: % Blasts, flow cytometry



Blood analysis: Absolute counts (G/L)

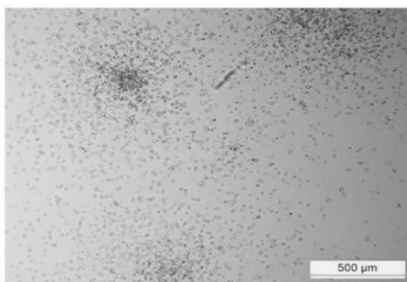


Case 2: ARGX-110/Aza combo reduces AML stemness

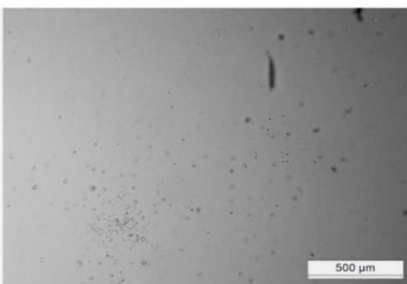
ARGX-110 monotherapy reduces LSCs outgrowth

White light microscopy (5,000 cells)

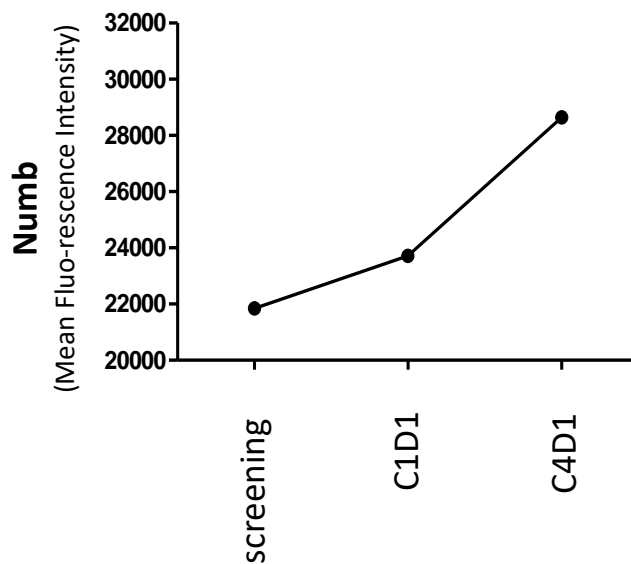
Screening



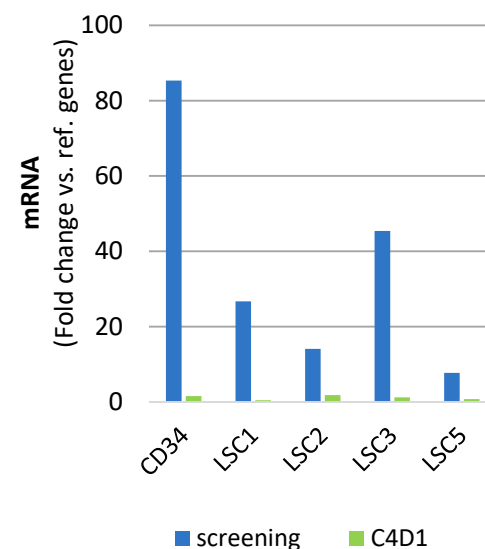
ARGX-110: Cycle 1 Day 1



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

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)

Business development & financials







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 all R&D through completion of IND-enabling studies
- **argenx** retains rights to combine ARGX-115 with its pipeline programs

Financial Highlights

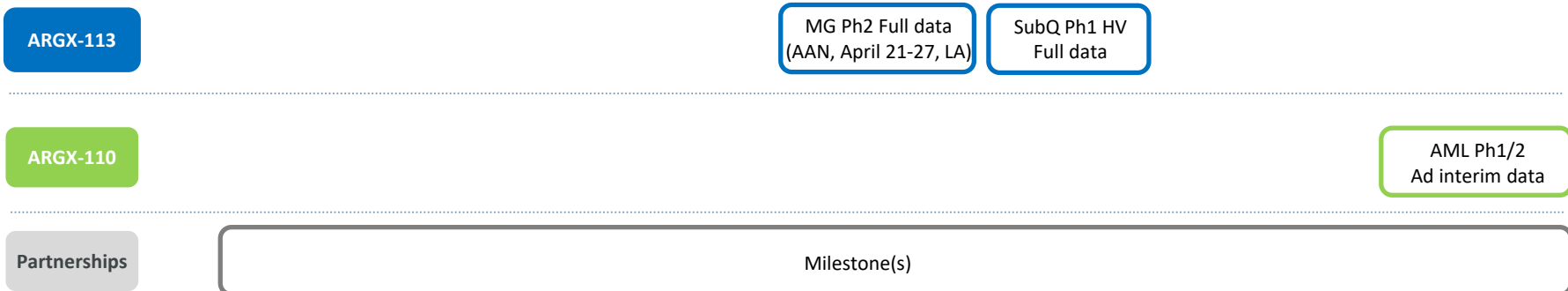
- **\$40mm upfront payment**
- **Received** first of two **\$10mm** preclinical milestones
- **\$625mm in potential** development, regulatory and commercial milestones
- **Tiered royalties** on sales at percentages ranging from **mid-single digits to low teens**
- **Co-promotional** rights for ARGX-115-based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

The image shows two overlapping circles. The top circle contains the argenx logo, which consists of the word "argenx" in a sans-serif font followed by a green checkmark icon. The bottom circle contains the abbvie logo, which is the word "abbvie" in a lowercase sans-serif font.

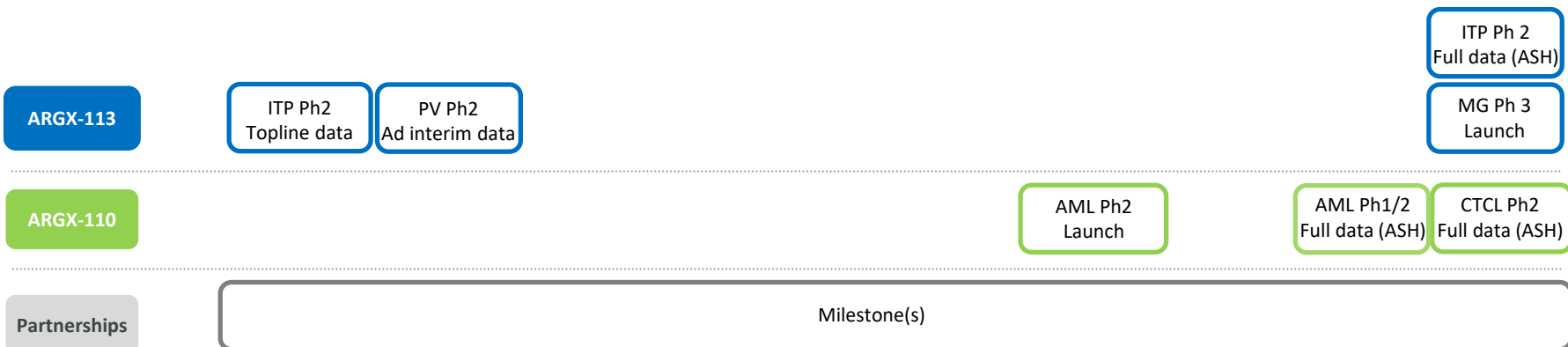
Partner	Asset	Key commentary
	ARGX-109 (Gerilimzumab)	<ul style="list-style-type: none"> Focused on developing an anti-IL-6 antibody for Rheumatoid Arthritis Bird Rock responsible for all costs incurred in R&D and commercialization
	ARGX-112	<ul style="list-style-type: none"> Focused on inflammation-based 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
	ARGX-116	<ul style="list-style-type: none"> 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
Broteio Pharma	Undisclosed	<ul style="list-style-type: none"> Focused on developing a differentiated antibody against a novel complement target Potential to act synergistically with ARGX-113 Jointly responsible for development expenses until preclinical POC — argenx granted exclusive option to license program after achieving preclinical POC
	Discovery Programs	<ul style="list-style-type: none"> 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

Key Upcoming Milestones & Communications

2018 Q1 Q2



2018 Q3 Q4

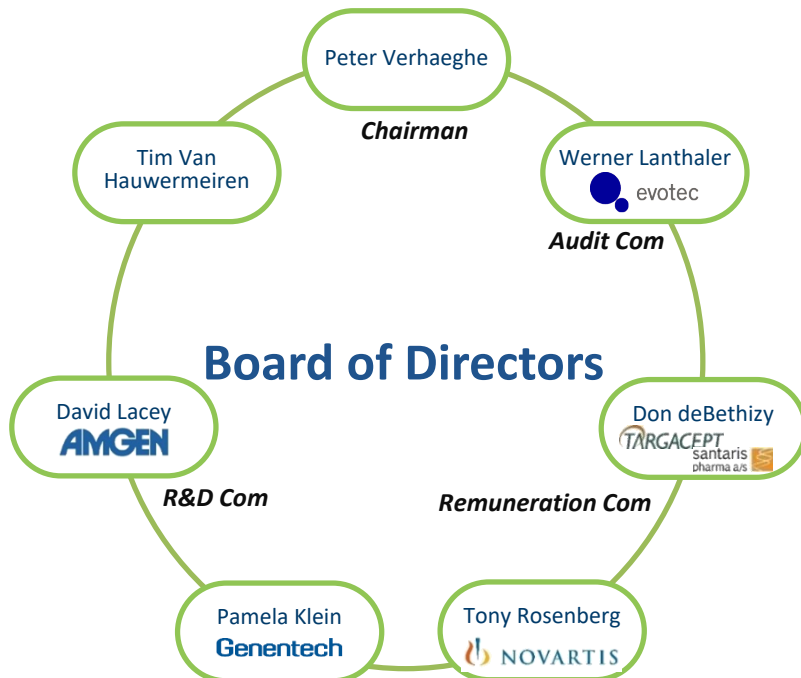
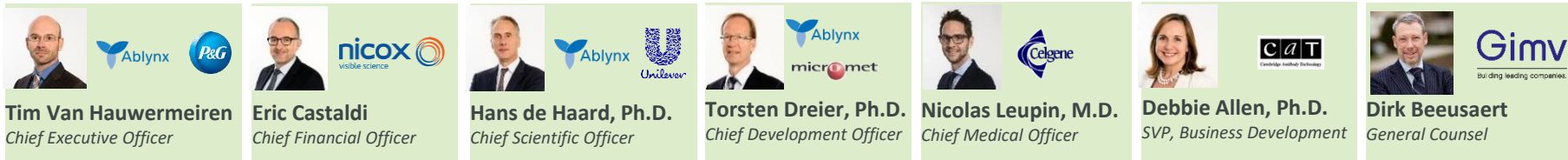


Appendix

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Management



Thank you!
