Creating value from highly differentiated antibody therapeutics

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Introduction

Creating value from highly differentiated antibodies

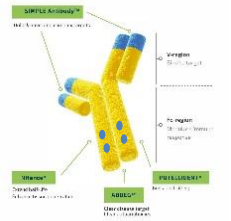
Rich proprietary pipeline

- Cancer & severe autoimmune diseases
- 4 products in clinical phase

Thriving strategic alliances

- Validation by industrial partners
- Access to novel targets via Innovative Access Program

Competitive technology suite



- Antibodies with differentiated modes of action
- Based on llama immune system and unique Fc engineering

Strong financials

- Strong cash position - € 103Mio Sept 2016
- Supported by blue-chip biopharma investors
- > € 2B potential future income from partnerships

Business model maximizing shareholder value

Generating differentiated antibody candidates...

Novel Targets + **argenx Technology Suite** =  **Maximally differentiated mAbs**

...capturing value at optimal stages

Discovery	Preclinical development		Early & late clinical development		
Platform deals	Product deals outside strategic focus		Product deals large indications		Product portfolio progress to clinical PoC	
Shire ✓	ARGX-109 (Bird Rock Bio) ✓		ARGX-115 (abbvie) ✓		ARGX-113 (Ph2 2017) ☐	
BAYER ✓	Undisclosed (LEO) ✓		ARGX-111 ☐		ARGX-110 (Ph2 2017) ☐	
✓						

Major value inflection point

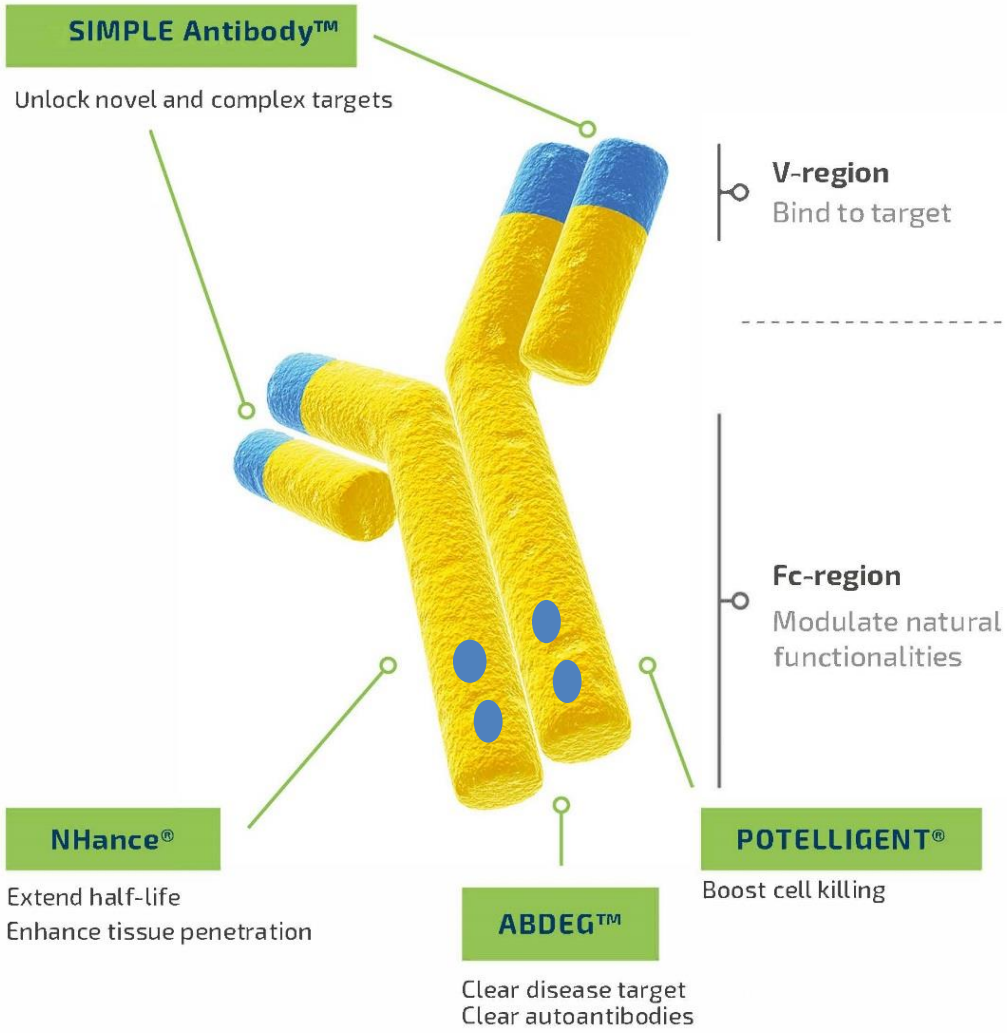
Proprietary pipeline in cancer and severe autoimmunity

		Drug candidate	Target	Indication	Pre-clinical / Phase 1 / Phase 2
Autoimmune diseases		ARGX-113	FcRn	Myasthenia Gravis	
				Immune Thrombocytopenia	1Q 2017
				SubQ dosing HV	2H 2017
Cancer immunotherapy		ARGX-110	CD70	Acute Myeloid Leukemia	
				T-Cell Lymphoma	1Q 2017
Metastatic cancer		ARGX-111	c-MET	Solid tumors Blood cancer	
		Discovery Undisclosed		Multiple	
Partnered, non-dilutive income	abbvie	ARGX-115	GARP	Cancer Immunotherapy	
	BIED ROCK BIO	ARGX-109 Gerilimzumab	IL-6	Autoimmunity	
	LEO	ARGX-112	IL-22R	Skin inflammation	
	Shire	Undisclosed		Undisclosed	

SIMPLE Antibody™ platform & Fc Engineering

Unique technology platform

Augmenting antibody natural functionalities



Monoclonal antibodies: unique therapeutic opportunities

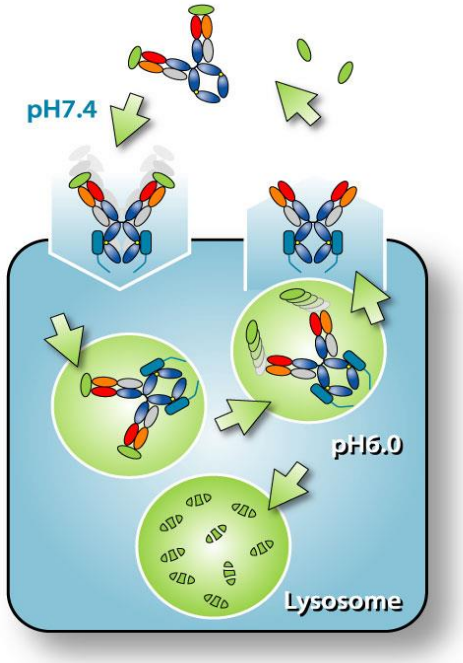
Combining SIMPLE Antibody™ and Fc engineering technologies → **augments antibody natural functionalities**

Continuous technology innovation

Antibody mediated target clearance

NHance®/ABDEG™
FcRn modulation

SIMPLE ANTIBODY™
pH-dependent target binding



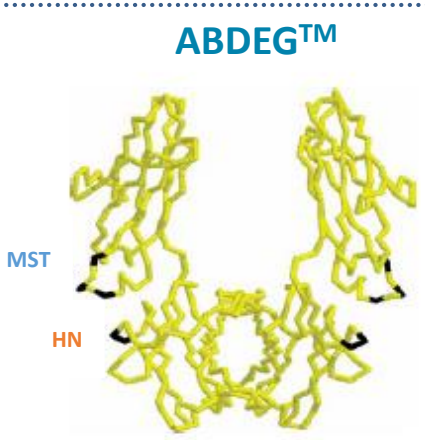
- Clinical potential for indications:
  - with high circulating target concentrations
  - which require fast target clearance
  - e.g. inflammatory cytokines (receptors)

ARGX-113: Advancing to clinical proof of concept

ARGX-113: Lead program targeting autoimmune diseases

Mechanism of action – antagonizing FcRn

Proprietary Fc mutations

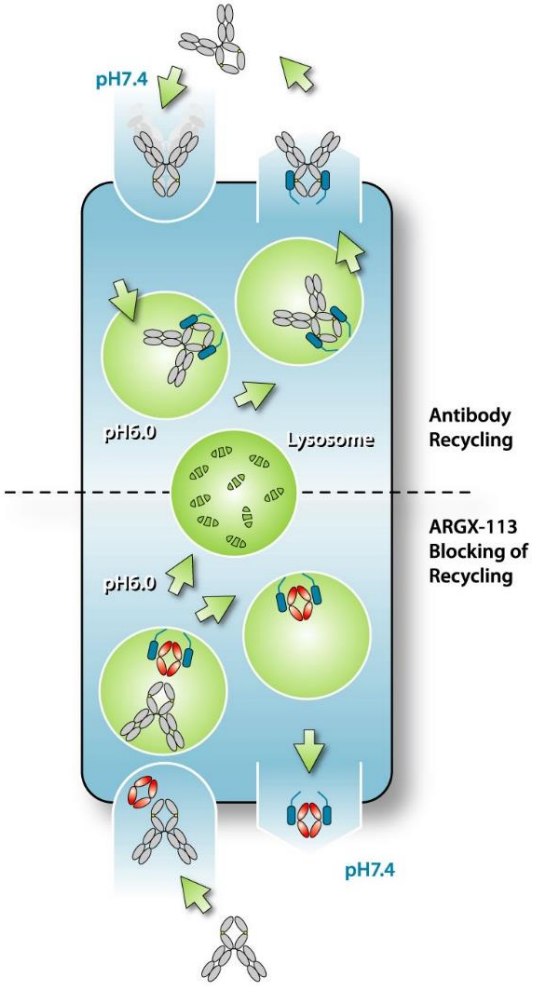


Engineering the Fc region of immunoglobulin G to modulate *in vivo* antibody levels

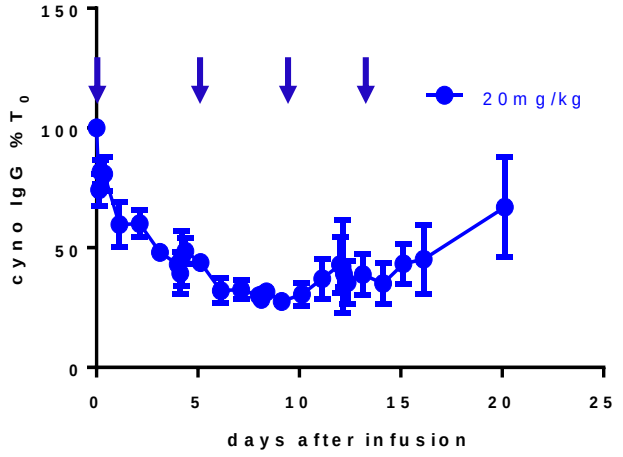
Carlos Vaccaro[1], Jinchun Zhou[1], Raimund J Ober[1,2] & E Sally Ward[1]

NATURE BIOTECHNOLOGY VOLUME 23 NUMBER 10 OCTOBER 2005

Block IgG recycling



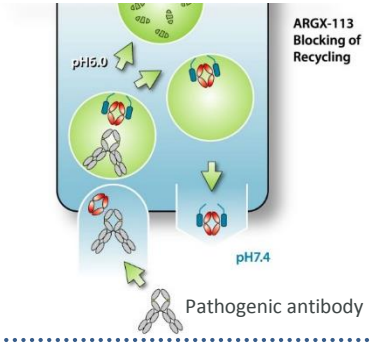
Repeat dose ARGX-113



- Saturation of PD effect at doses ≥ 20 mg/kg
- Repeat dosing > single dose

ARGX-113: Pipeline-in-product opportunity

Prioritizing IgG mediated diseases



Solid biology rationale
Pathogenic IgG's proven to mediate disease

Feasible for biotech
Orphan potential
Economically viable
Clinical & Regulatory path clear

High proof of concept value
Spill-over effect into adjacent indications

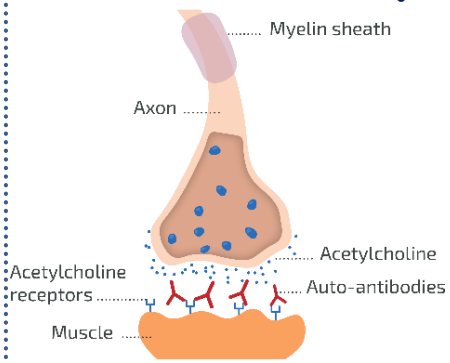
- Myasthenia Gravis
- Immune Thrombocytopenia
- Pemphigus
- Bullous Pemphigoid
- Epidermolysis Bullosa Acquisita
- Scleroderma
- Anca Vasculitis
- Lupus
- Multiple Sclerosis
- Rheumatoid Arthritis
- ...

Source: argenx data

Myasthenia gravis: Fact sheet

bad life-threatening choking muscle dislocation eye eyelid fatigued pain seeing swallowing talking tired weakness trouble walk weight

- Rare disease
- Prevalence (US): 47,000 patients with generalized MG
- All ages & both genders



- Caused by auto-antibodies (auto-IgG's) destroying neuromuscular junction:
  - Blocking of AChR
  - Cross-linking + internalization
  - Complement recruitment

- Treatment options:
  - Corticosteroids & immunomodulatory agents
  - IVIg or Plasmapheresis
  - Treatment cycles back and forward
- Side effects: cancer cataract diabetes disfigurement fibrosis headache hematological suppression hypertension infection injury liver malignancy mood osteopenia osteoporosis pulmonary renal teratogenicity thrombosis weight

- ARGX-113
  - Potential breakthrough approach to clear pathogenic auto-IgG's
  - Addressing unmet need (cf MG task force)
    - Elimination of patient symptoms → achieve remission
    - Minimizing side effects of medication

" ARGX-113 has the potential to improve initial response to steroids, provides option for chronic managment and rapidly controls flares", James Howard, M.D., University of North Carolina at Chapel Hill

Autoantibody levels (IgGs) correlate with disease state in MG

30-60 % autoantibody reduction clinically meaningful

Treatment*	Plasmapheresis	Immunoadsorption	IVIg
Decrease in autoantibody levels (%) after treatment	62.2 ± 6.3	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

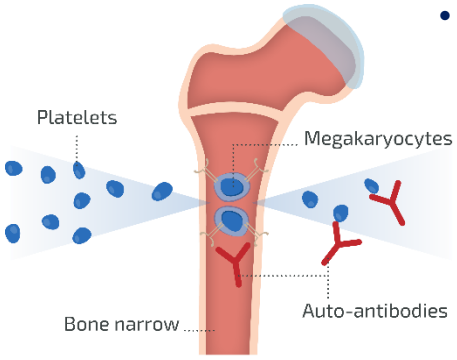
Liu et al. 2010, Ther Apher Dial

- Degree of autoantibody reduction: correlates with clinical improvement & reduced hospital stay
- Similar observations reported for other autoimmune disorders
- ARGX-113: potential to provide stronger IgG reduction

Immune thrombocytopenia: Fact sheet

Fatigue Emotional strain Impact on work Fear of bleeding Impact on social activities Bruising

- Rare disease
- Prevalence (US): 50,000 patients
- Female > male
- Highest incidence > 60 years of age



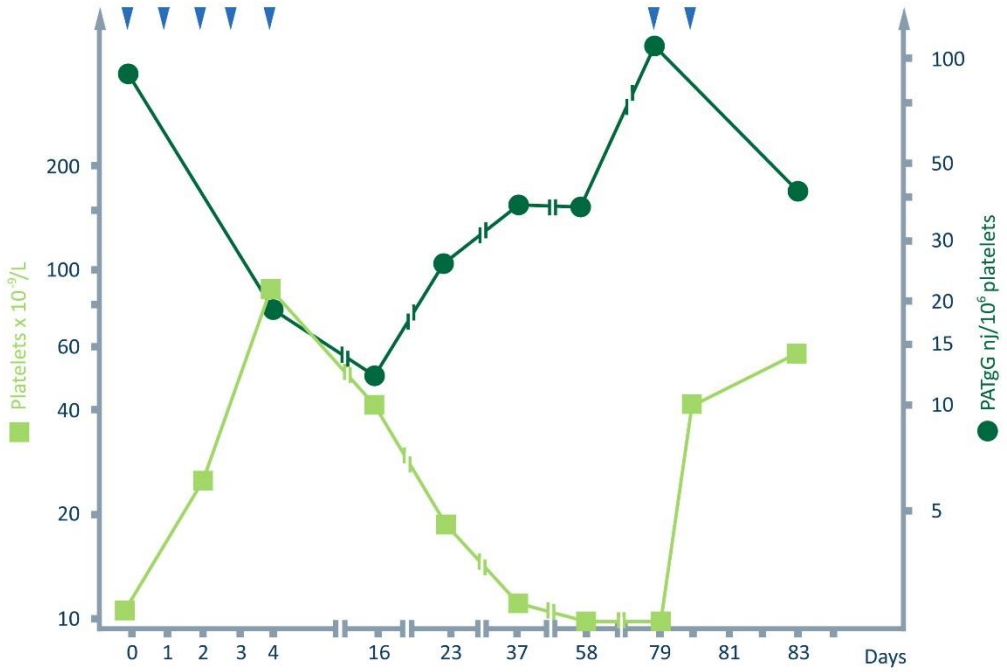
- Caused by auto-antibodies (auto-IgG's) destroying blood platelets:
  - Increased platelet removal
  - Reduced platelet production

- Treatment options:
  - Multiple iterations on corticosteroids & IVIg
  - Immunomodulatory agents
  - TPO-mimetics & splenectomy
- Side effects:

anaphylaxis anorexia backache cancer cataracts depression diabetes fatal hemolysis hepatitis hypertension infection infusion reactions leukoencephalopathy nausea osteoporosis psychosis renal sweating neutropenia thrombosis vomiting weakness

- ARGX-113
  - Potential breakthrough approach to clear pathogenic auto-IgG's
  - Addressing unmet need
    - Elimination of patient symptoms → achieve remission
    - Minimizing side effects of medication
  - Potential use in patients with inadequate response to steroids & in place of IVIg before second line agents

Autoantibody levels (IgGs) correlate with ITP disease score



▼ = IVIg treatment

● = AutoAb

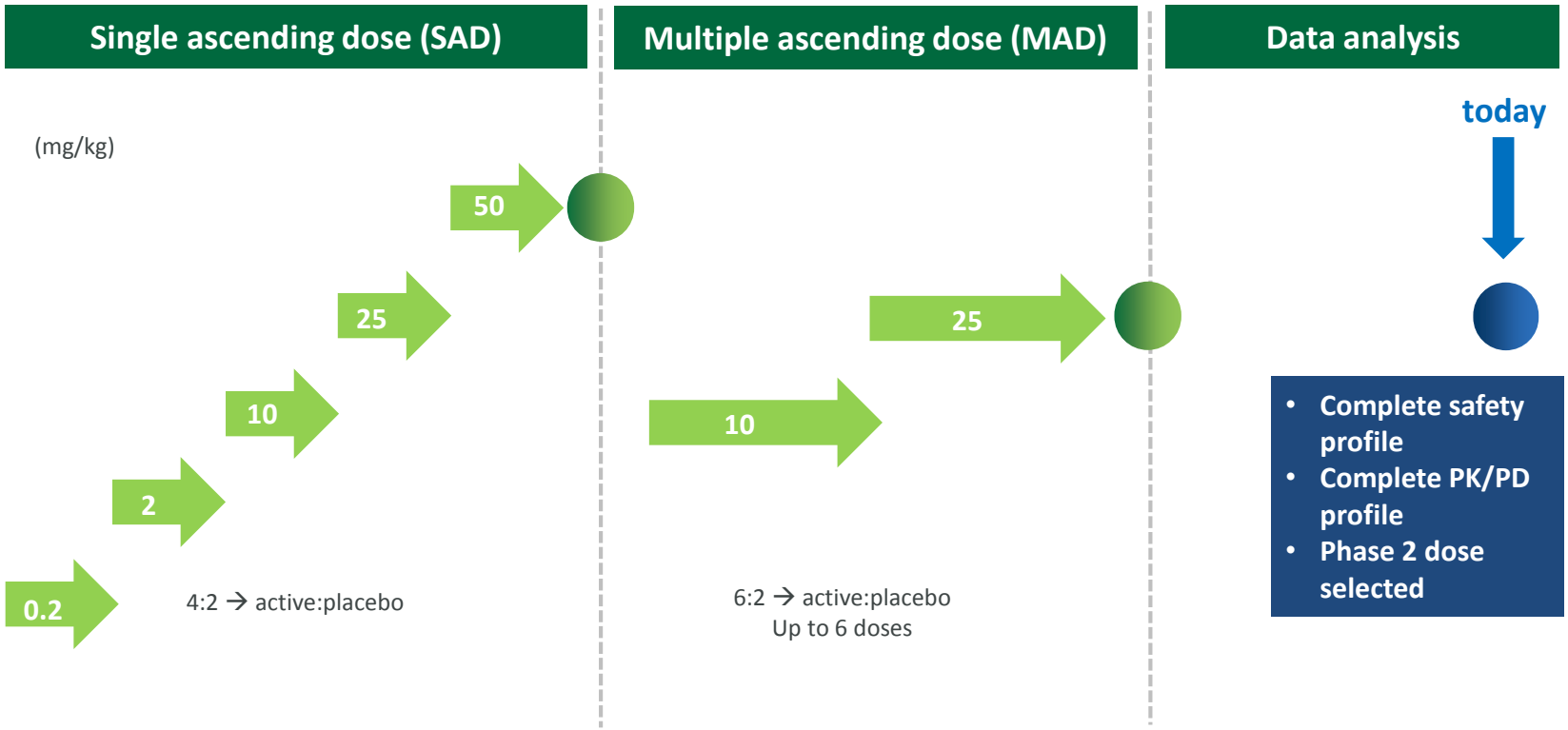
■ = Platelet counts

- ITP: autoantibodies inhibit platelet production and accelerate platelet destruction
- IVIg, plasmapheresis and immunoadsorption: proven clinical efficacy in ITP
- MoA IVIg: lowering autoantibodies results in platelet increase
- Plasmapheresis and immunoadsorption: identical MoA (data not shown)

Winiarski et al. 1983, Scand J Haematol

ARGX-113: Favorable safety and tolerability profile observed

Phase I study design & status

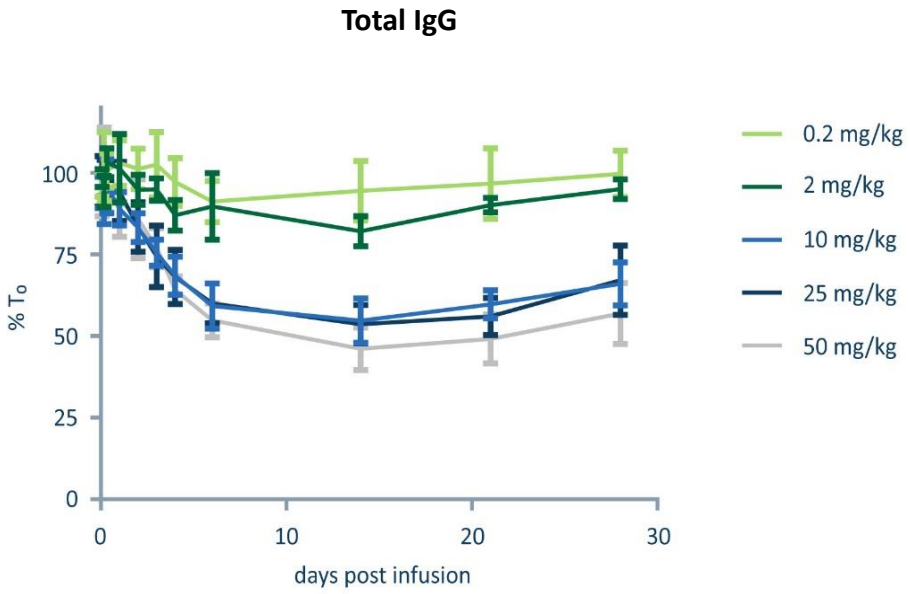


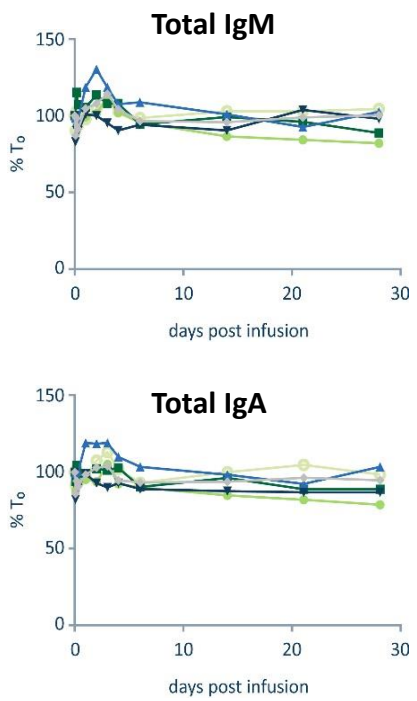
- Double-blind, placebo-controlled study in healthy volunteers
- SAD & MAD dosing completed according to plan (62 healthy volunteers in total)
- Favorable safety and tolerability profile observed

Source: argenx data

ARGX-113: Selective IgG reduction

Single ascending dose escalation study (SAD) in healthy volunteers





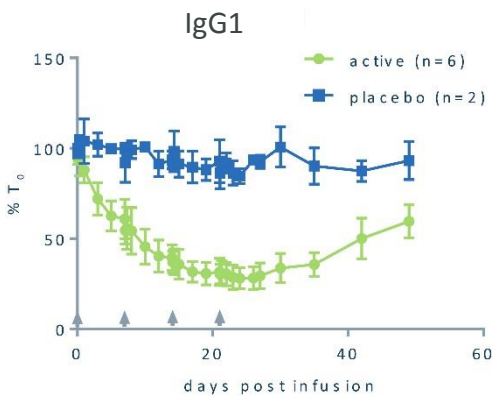
- Single 2h infusion: selective IgG reduction, not affecting IgM/IgA and albumin levels (not shown)
- Maximal PD effect (~50% IgG reduction) as of 6 days after infusion
- Low IgG levels maintained for several weeks
- Saturation of PD effect as of 10 mg/kg dose

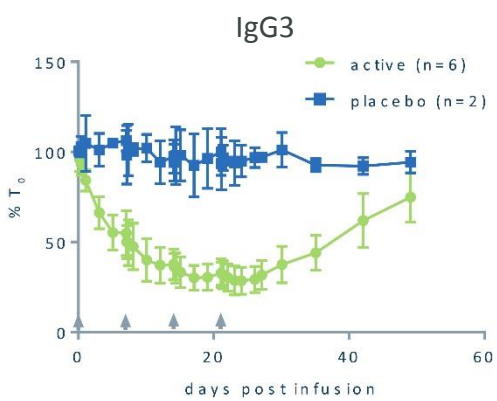
Source: argenx data – blinded, uncleaned from HV study

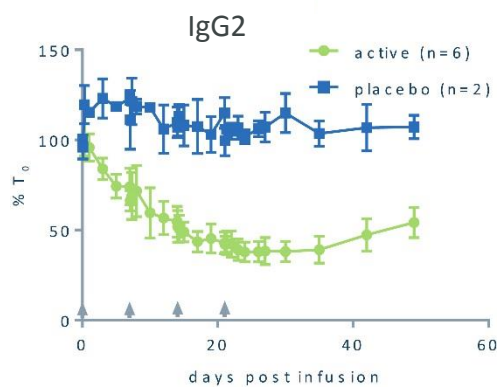
ARGX-113: Potent reduction of IgGs across isotypes

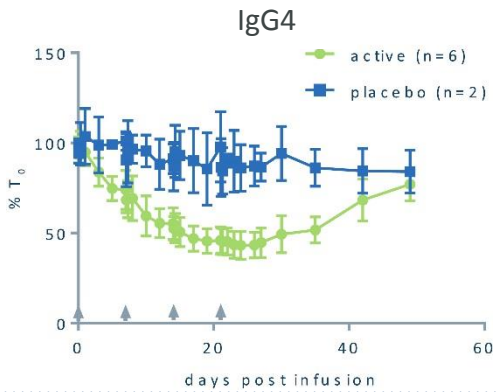
PD data multiple ascending dose (MAD) study

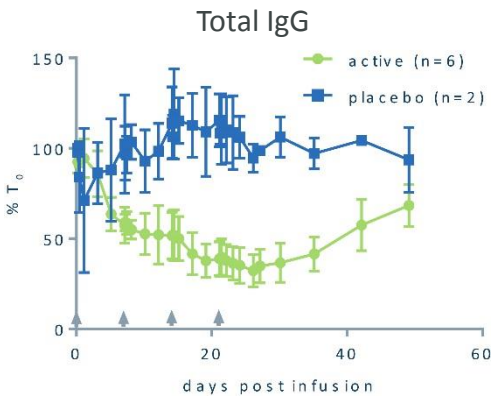
Dosing: 10 mg/kg, every 7 days









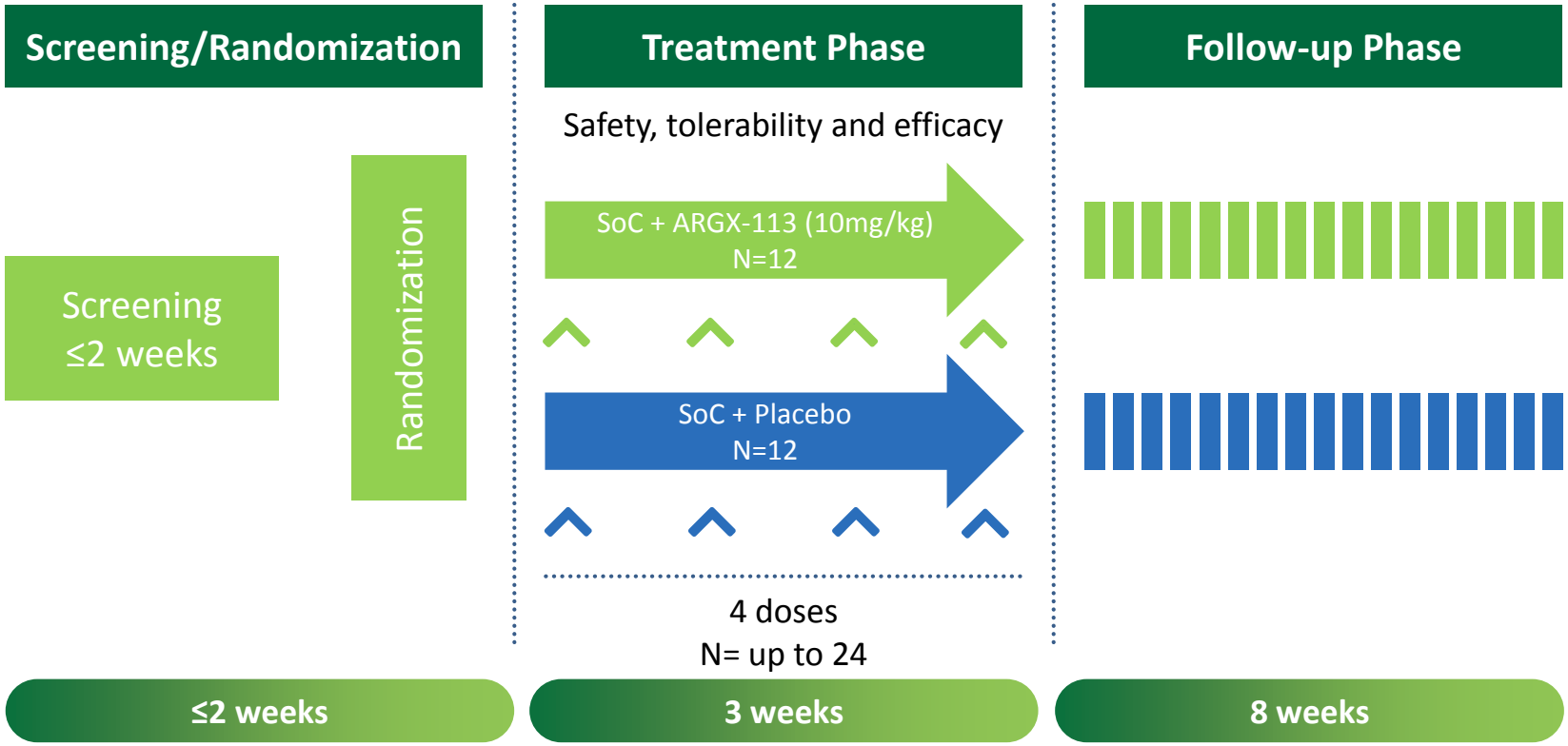


- Clinically meaningful IgG reduction: 50% achieved in 1 week; up to 85% maximum reduction
- After the last dosing, IgG levels remain reduced by 50% or more for a period of 3 weeks
- After the last dosing, IgG levels return to baseline in > 1 month
- Comparable data for 25 mg/kg, every 7 days (data not shown)

Source: argenx data – blinded, uncleaned from HV study

ARGX-113 in MG: Phase II trial design

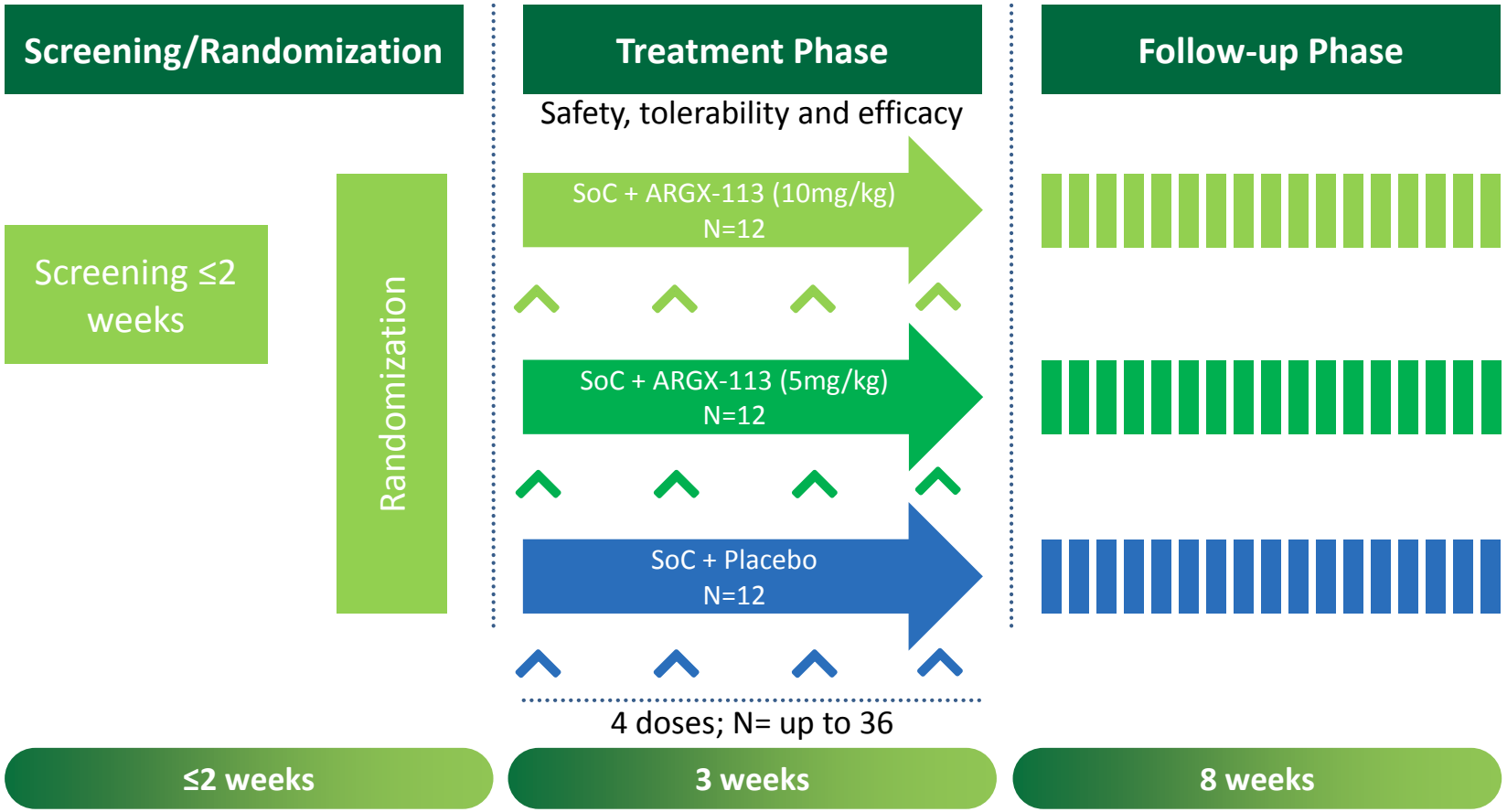
- Population: Autoimmune MG patients with generalized muscle weakness with total MG-ADL score ≥ 5 with more than 50% of this score attributed to non-ocular items



- Primary Objectives: Evaluate safety and tolerability
- Secondary Objectives: Evaluate efficacy, impact on quality of life and immunogenicity
  Assess pharmacokinetics (PK) pharmacodynamic (PD) marker

ARGX-113 in ITP: Phase II trial design

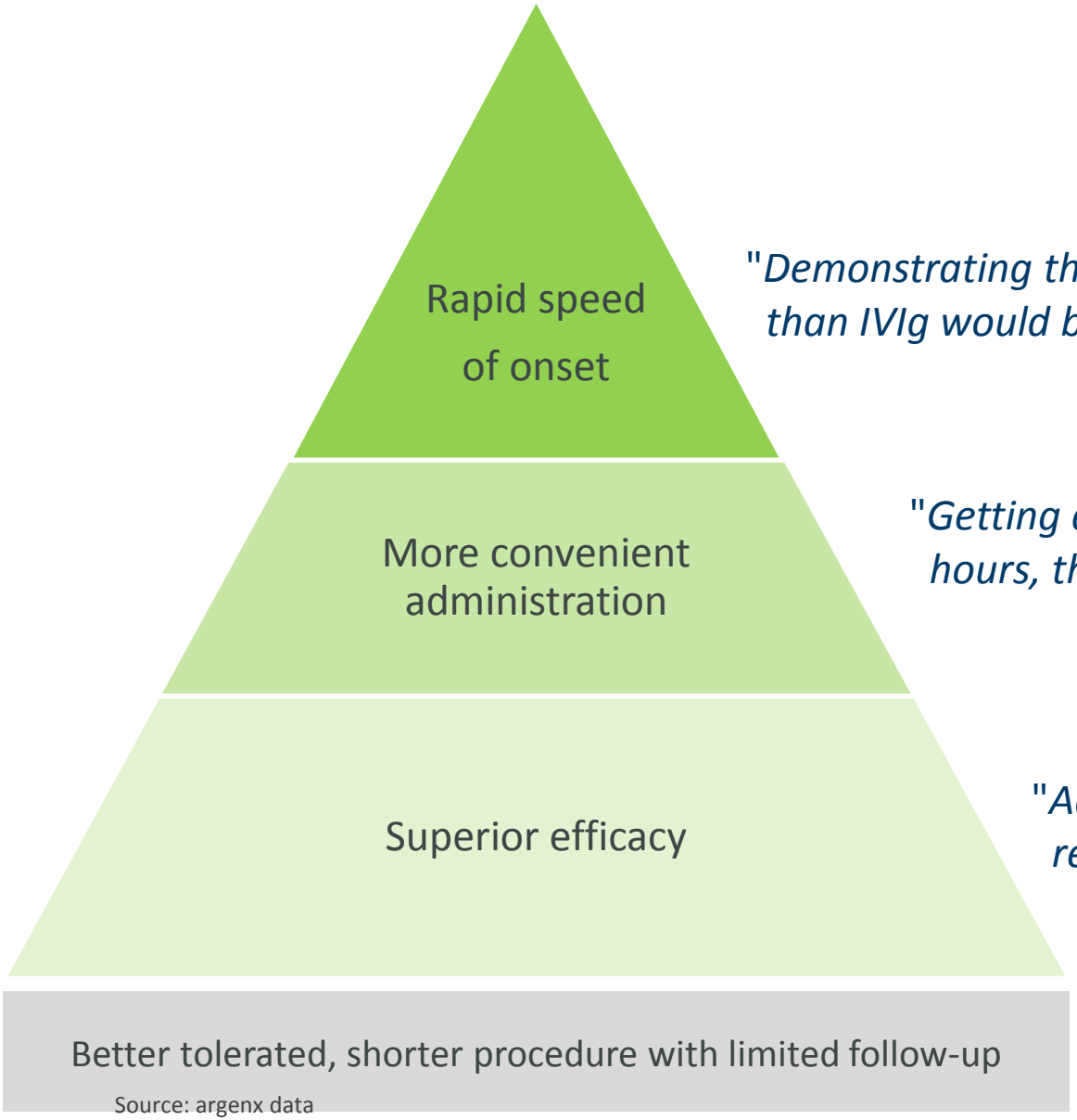
- Population: ITP patients with platelet levels < 30 X 10^9/L



- Primary Objectives: Evaluate safety and tolerability
- Secondary Objectives: Assess effect on platelet counts and on use of rescue treatment
  Assess pharmacokinetics (PK) and pharmacodynamic (PD) effect
  Evaluate immunogenicity

ARGX-113 vs IVIg/PLEX

Key differentiators for MG



"*Demonstrating that its onset of action is faster than IVIg would be fantastic,*" MG KOL

"*Getting an infusion done within 2 hours, that is an attractive piece*“ MG KOL

"*Acute MG crisis, I don't think it responds all that well to IVIg*," MG KOL

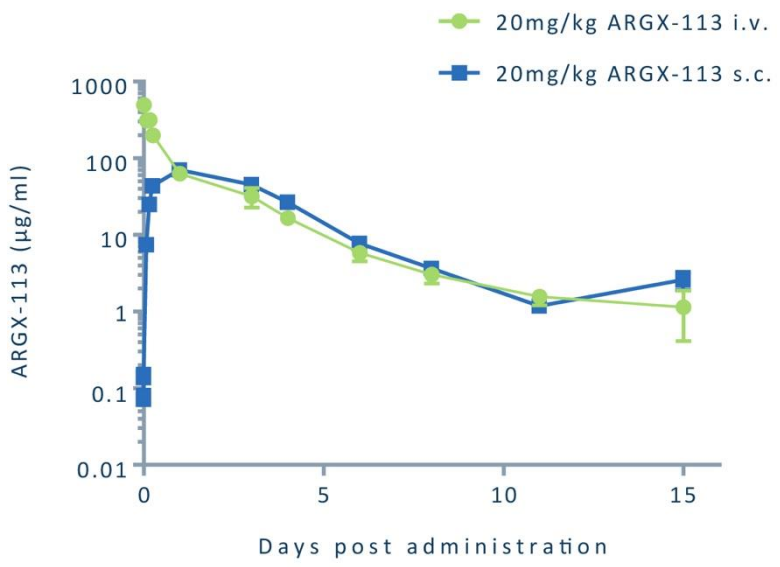
Source: argenx data

ARGX-113-s, further development

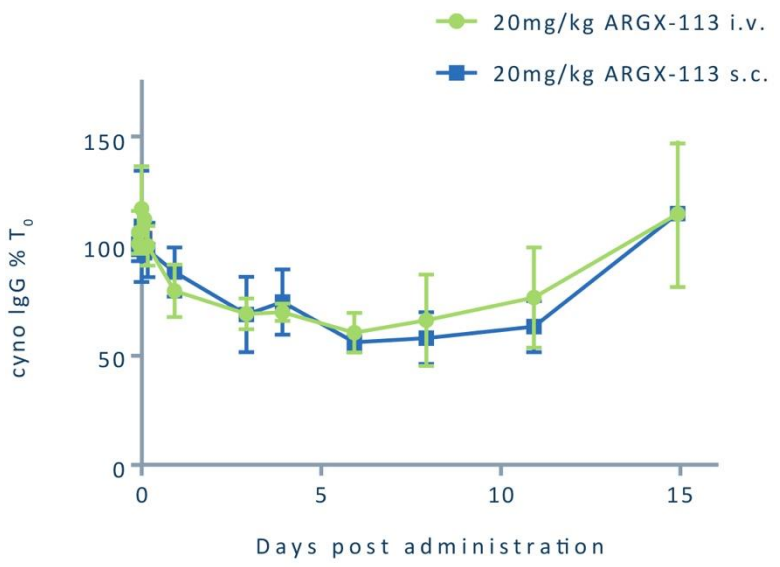
ARGX-113: Feasibility subQ dosing proven

Cynomolgus PoC: comparable PD and PK profiles for IV and subQ administration

PK single dose administration: IV vs subQ



PD single dose administration: IV vs subQ



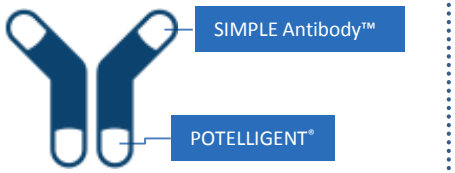
- IV versus subQ dosing:
  - Comparable half life
  - Favorable bio-availability of the compound in subQ dosing
  - Comparable reduction of IgG's with single dose; up to 50%

Source: argenx data

ARGX-110: Phase I/II mono & combo therapy

ARGX-110: targets CD70

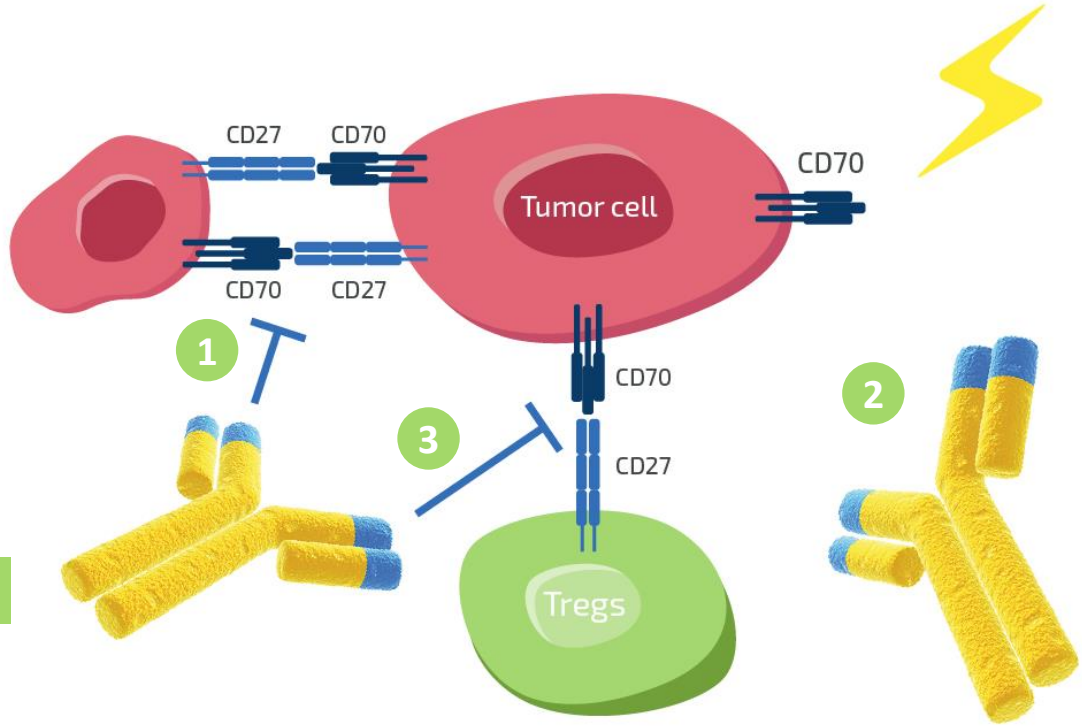
3 distinct modes of action to address tumor cell



1. Block tumor growth signal

2. Kill tumor

3. Restore immune surveillance

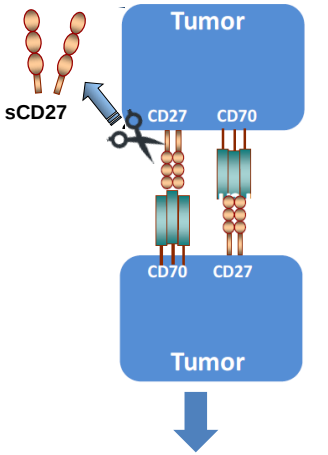


Prof. Ochsenbein won the 'Otto Naegeli Prize 2016', the most highly esteemed biomedical award in Switserland.
"Of particularly great importance was the discovery that the interaction of CD70 with CD27 and subsequent signaling events has great therapeutic potential for the development of new, original methods of cancer treatment using immunotherapy."

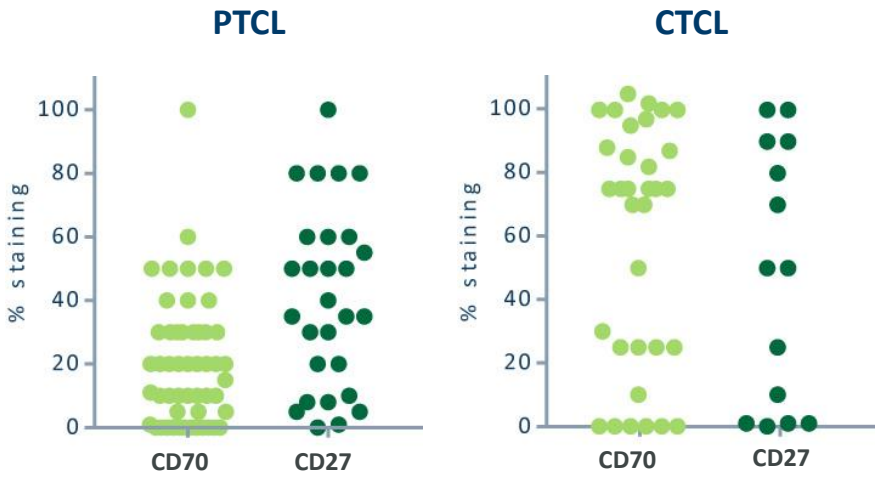
Silence et al. 2014, mAbs

ARGX-110: CD70/CD27 pathway highly relevant in TCL

CD70/CD27 on tumor cells

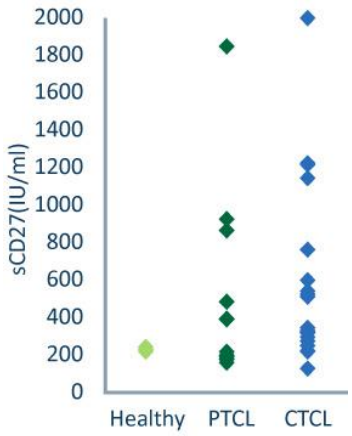


IHC of CD70/CD27 expression in TCL biopsies



Source: argenx data

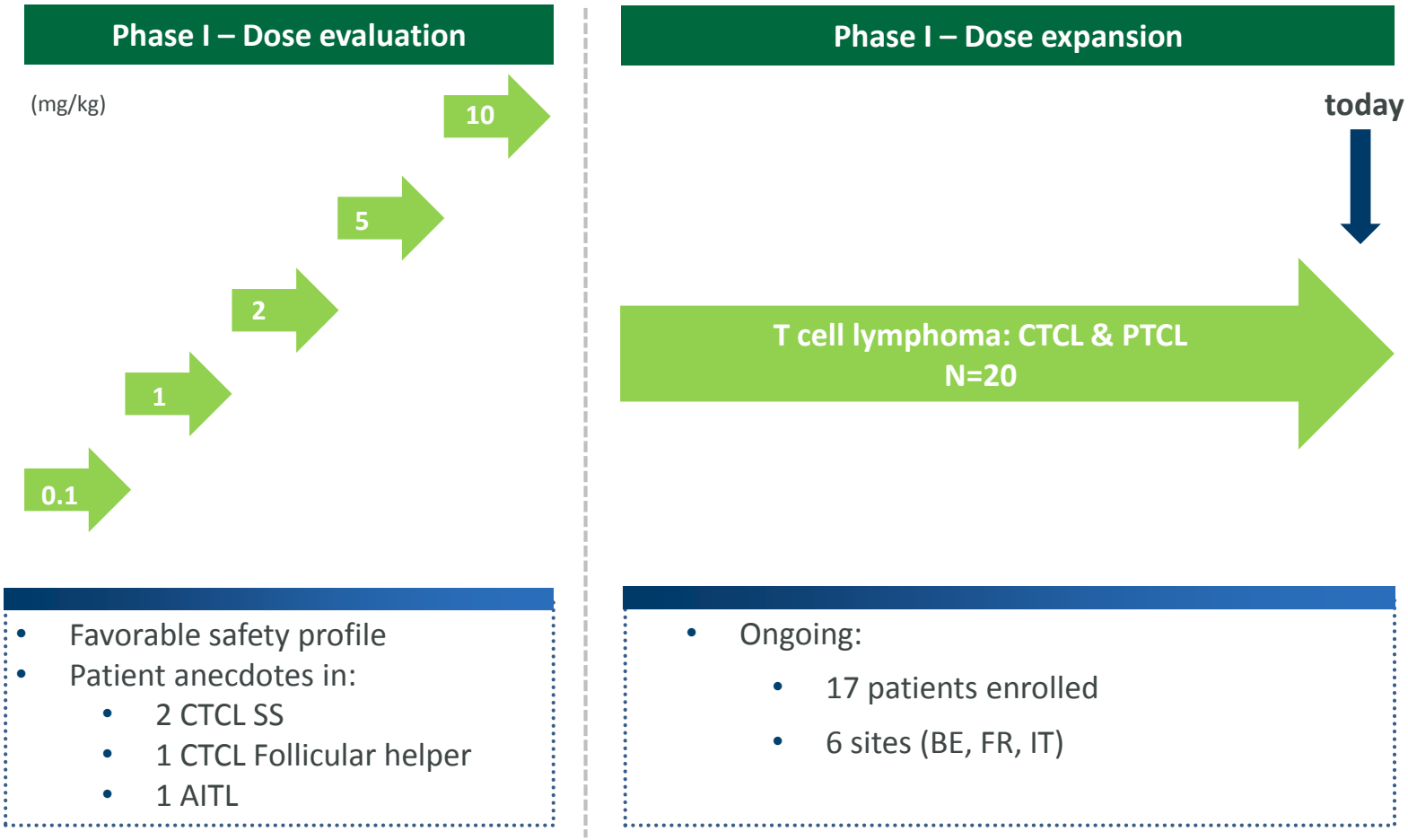
sCD27 levels in TCL patient



- CD70/CD27 strongly overexpressed across different TCL types
- Elevated sCD27 levels suggest strong pathway activity in TCL

Grewal et al. 2008, Expert Opin Ther Targets

ARGX-110: Phase I trial overview

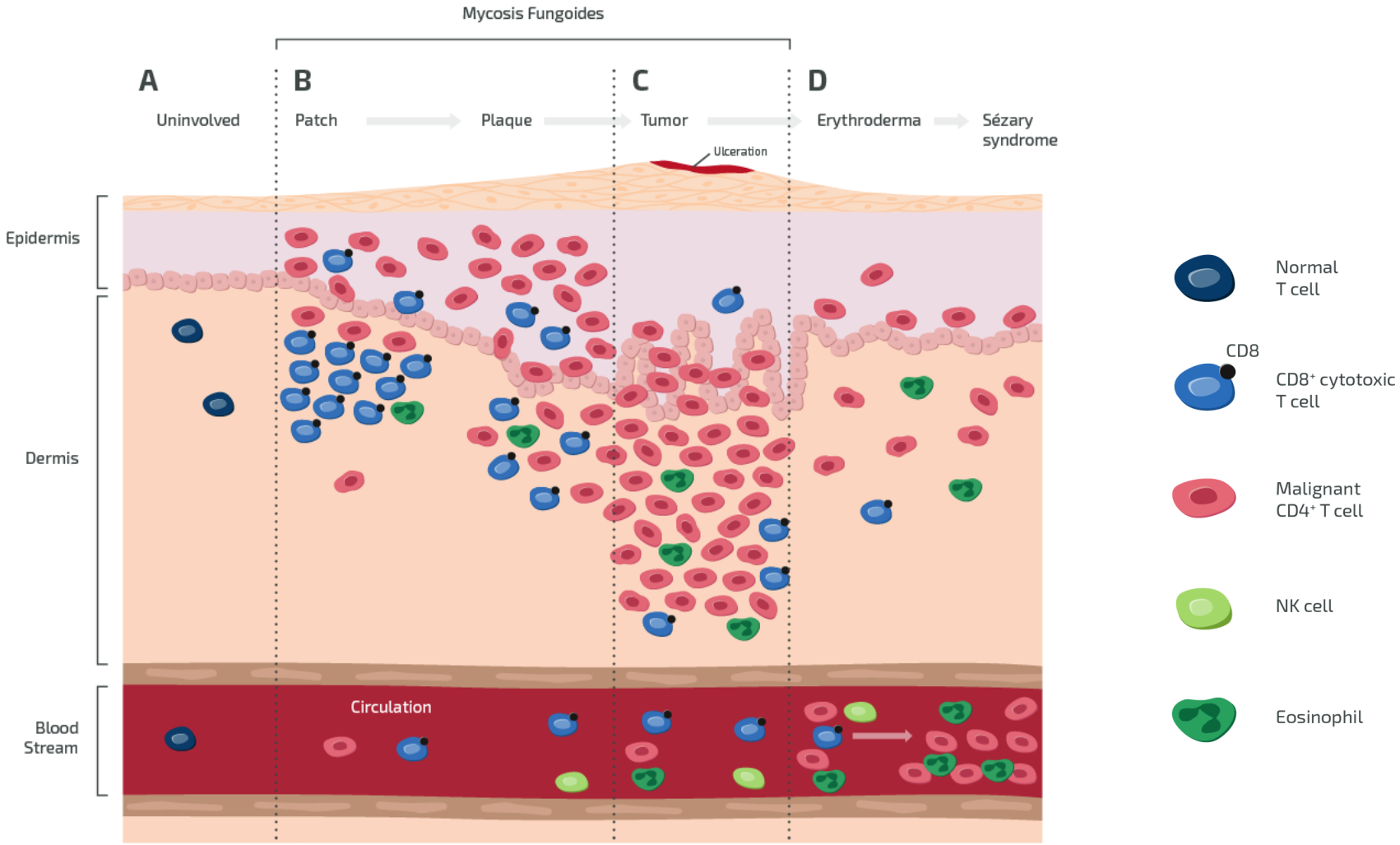


- Favorable safety profile
- Patient anecdotes in:
  - 2 CTCL SS
  - 1 CTCL Follicular helper
  - 1 AITL

- Ongoing:
  - 17 patients enrolled
  - 6 sites (BE, FR, IT)

Cutaneous TCL

Progression from Patch to Sézary Syndrome



Overview CTCL patients

ARGX-110 Phase Ib

CTCL in Expansion Cohort 2 (1 mg/kg q3w)

Indication	Stage	C1	C2	C3	C4	C5	C6	C7	C8	C9	C10	C11	C12	C20	Best response	
CTCL-MF	T4, N0, M0, B0														SD in skin, mSWAT ↑18%	
CTCL-MF/SS + (PTCL-NOS)	T4, N3, M0, B0														SD nodal, PD skin ↑63.5%	
CTCL-SS	T4, N3, M0, B1														PD?, mSWAT ↑4%	
CTCL-MF	T4, Nx, M0, B0														SD nodal and skin, mSWAT ↓42%	Patient 1
CTCL-SS	T4, Nx, M0, B2														PR in skin, SD nodal and blood, mSWAT ↓50% C4	Patient 3
CTCL-MF	T2, N0, M0, B0														PR in skin, mSWAT ↓62% C6	Patient 2
CTCL-SS	T2, Nx, M0, B2														PD	
CTCL-TFH like	T2, N0, M0, B0														PD in skin ↑56%	
CTCL-panniculitis like	T3, N0, M0, B0														PR in skin by PET/CT	Patient 4
CTCL-MF	T4, Nx, M0, B0														PD	

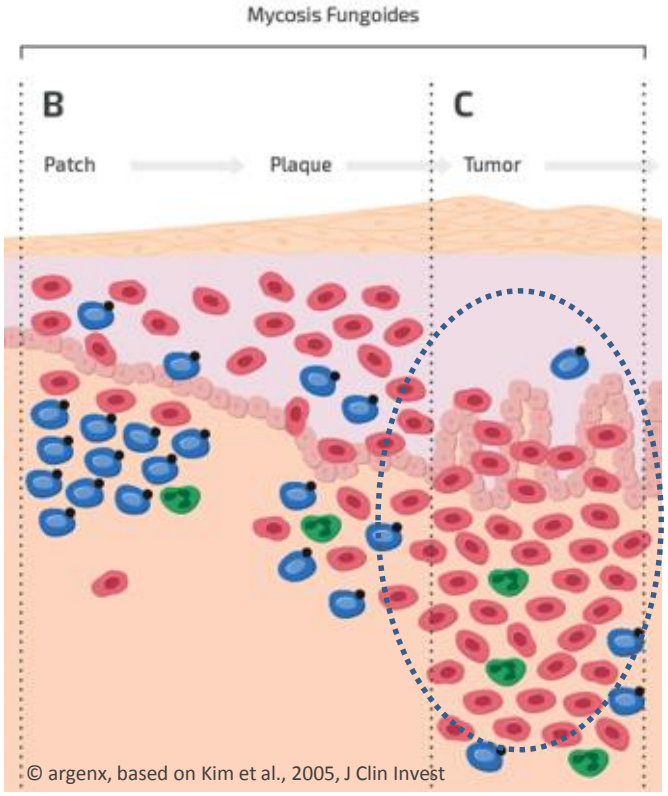
- Encouraging signs of clinical activity in expansion cohort 2: 2/10 SD and 3/10 PR
- Patients on study up to cycle 12
- 2/3 SD in dose escalation cohort

Source: argenx data - uncleaned

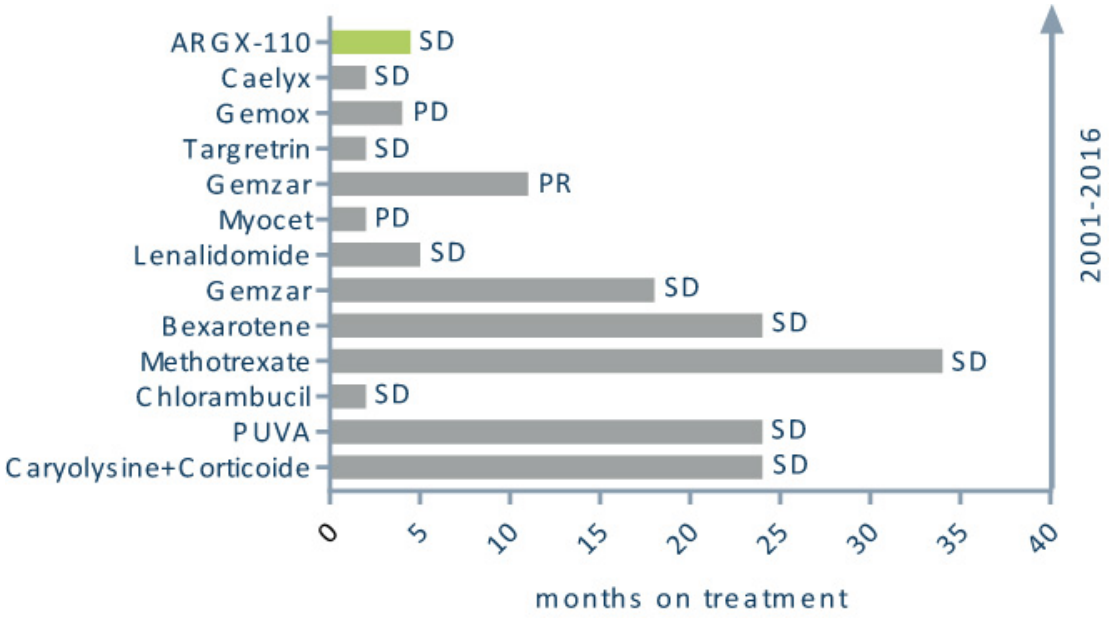
ARGX-110: Activity in heavily treated TCL patients

Patient 1: Cutaneous TCL – Mycosis fungoides (MF)

Patient	67 year old man with CTCL-MF, diagnosed on 21 Jan 2001
Tumor	Skin T4, Nx, M0, B0 (Stage IIIA)
Nr doses	6



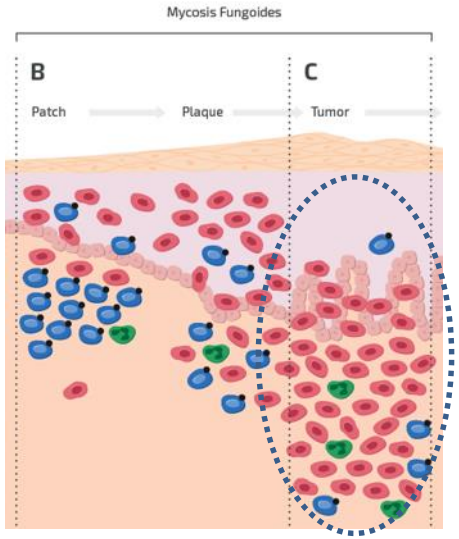
Treatments and best response



Source: argenx data –patient anecdotes - uncleaned

Malignant cells in the skin disappear after one dose

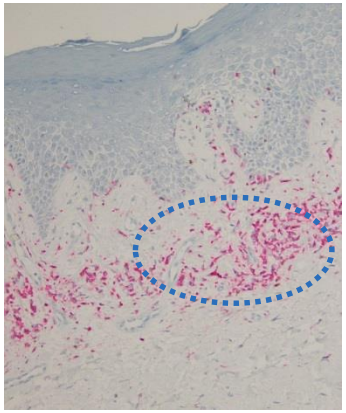
Patient 1: Cutaneous TCL – Mycosis fungoides (MF)



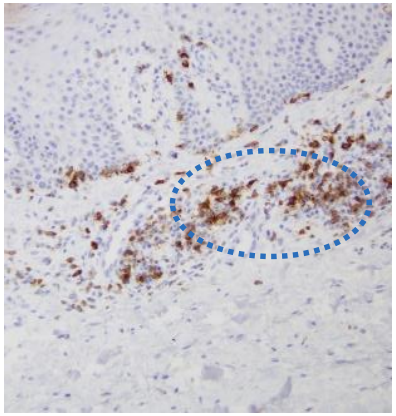
ARGX-110 treatment results in:

- decrease of CD4$^+$ malignant T-cells
- depletion of CD70$^+$ malignant T-cells
- infiltration of CD8$^+$ T-cells

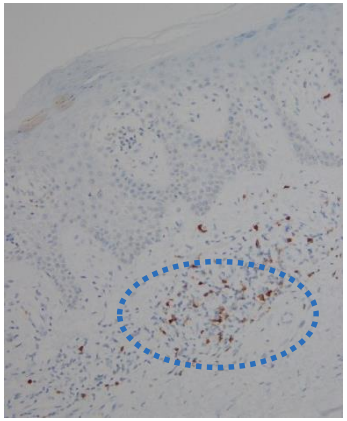
CD4$^+$ – pre



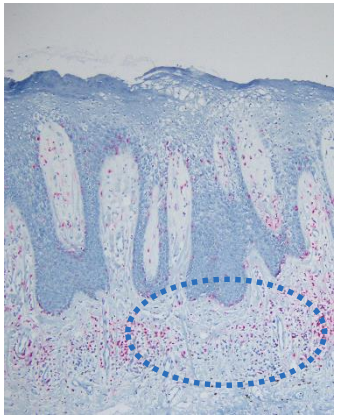
CD70 – pre



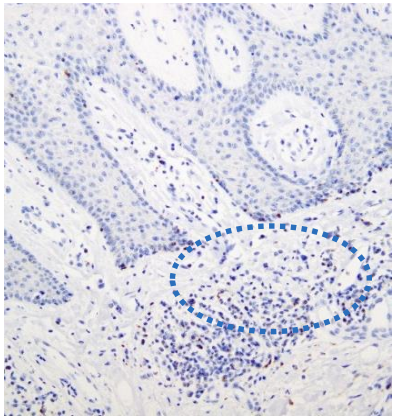
CD8$^+$ – pre



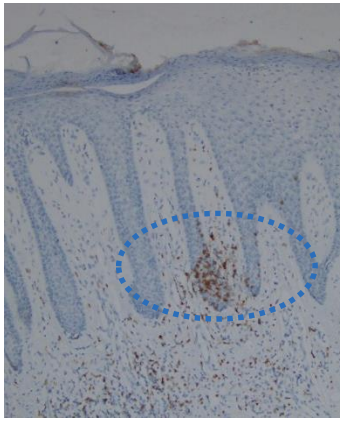
CD4$^+$ – post (C2)



CD70 – post (C2)



CD8$^+$ – post (C2)

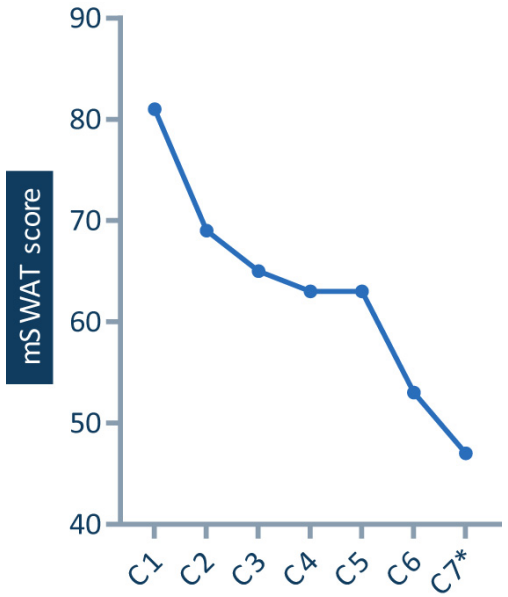


Source: argenx data –patient anecdotes - uncleaned

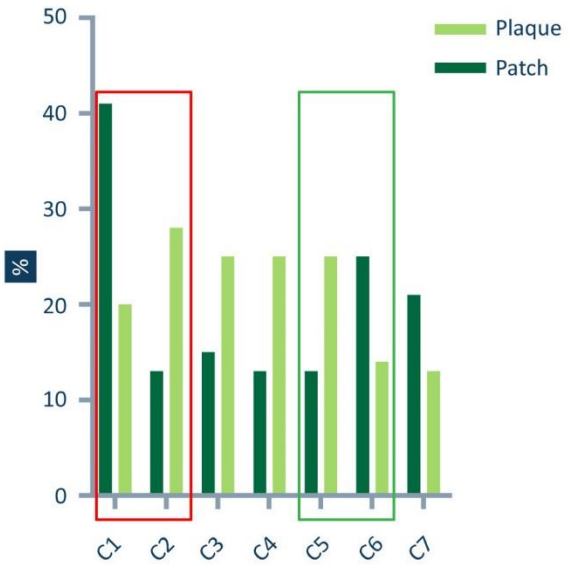
Reduction of mSWAT score and improvement of skin lesions

Patient 1: Cutaneous TCL – Mycosis fungoides (MF)

Reduced mSWAT score



Improvement of body lesions



mSWAT = modified Severity Weighted Assessment Tool
Plaque = raised or lowered flat lesions
Patch = flat lesions

- 42% reduction of mSWAT (C1 → C7)
- Cutaneous tumor lesions decrease in surface area C1→C2 (red box)
- Cutaneous tumor lesions improve from plaques to patches C5→C6 (green box)
- Patient experiences improved skin redness and itching & has decreased size of lesions

Olsen et al. 2007, J Clin Oncol

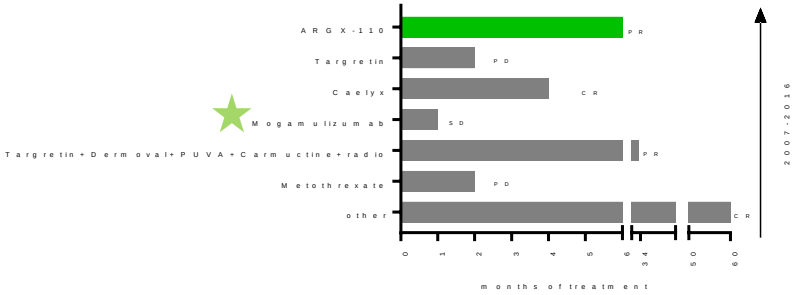
Source: argenx data –patient anecdotes - uncleaned

Partial response: improved mSWAT and skin lesions

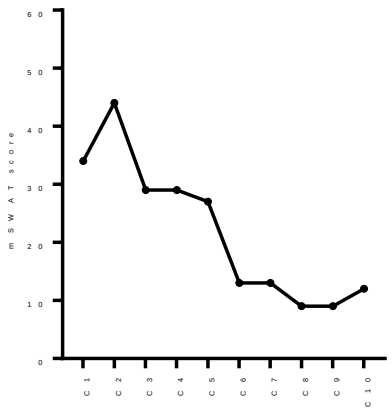
Patient 2 (2103): Cutaneous TCL – Mycosis fungoides (MF)

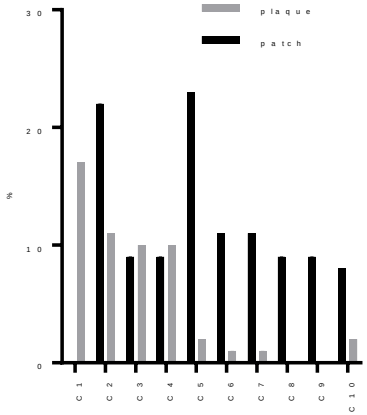
Patient	79 year old female with CTCL-MF, diagnosed 2007
Tumor	Skin T2, N0, M0, B0 (stage IB)
Doses	10, ongoing, 1 mg/kg q3w

Treatments and best response

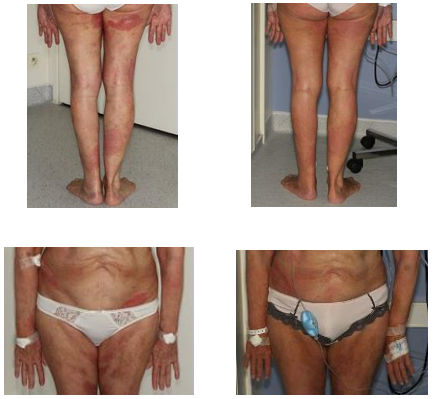


Reduced mSWAT score and total body lesions





Pre C1 C6



Pictures kindly provided by investigator

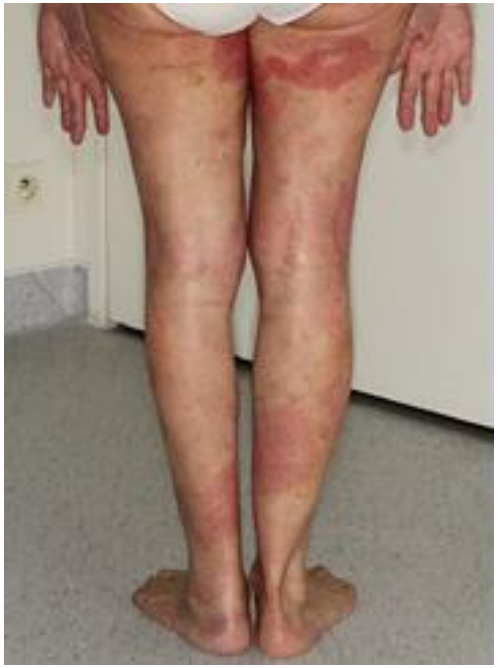
- Cutaneous tumor lesions decrease in surface area; 60% reduction of mSWAT and a partial response (PR)
- Cutaneous tumor lesions improve from plaques to patches

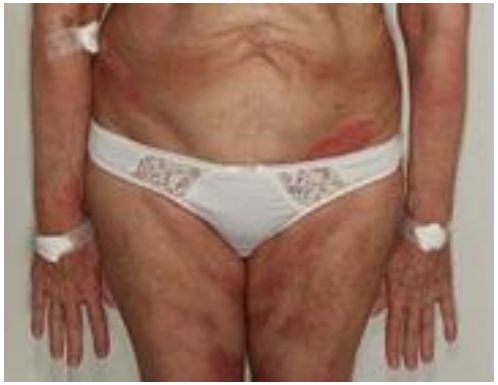
Source: argenx data –patient anecdotes - uncleaned

Partial response

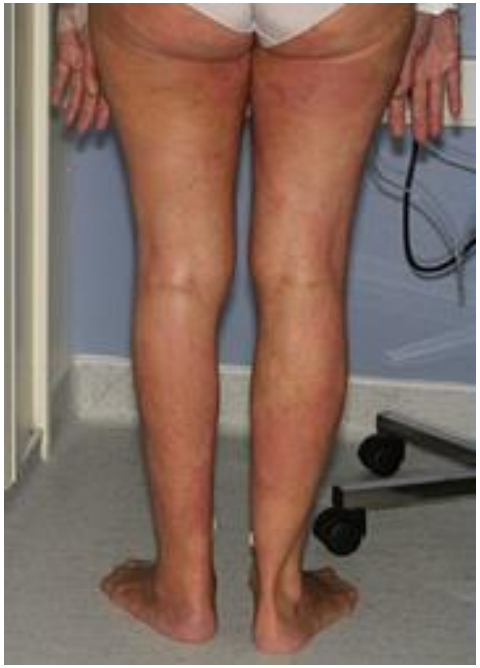
Patient 2: Cutaneous TCL – Mycosis fungoides (MF)

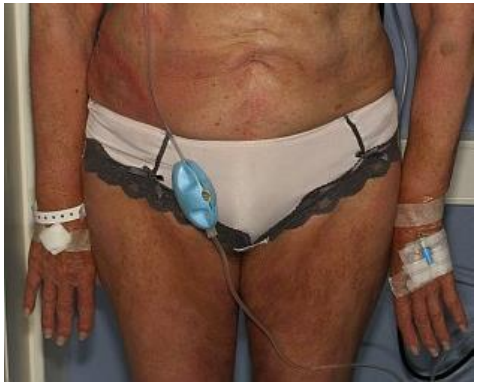
Pre - C1





Post - C6





Pictures kindly provided by investigator

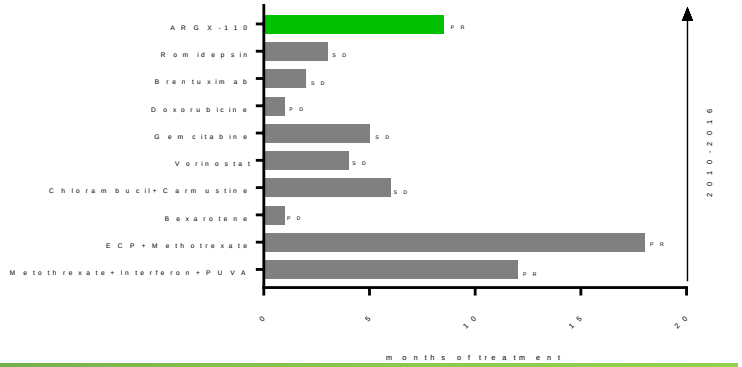
Source: argenx data –patient anecdotes - uncleaned

Stable disease: Improved mSWAT and skin lesions

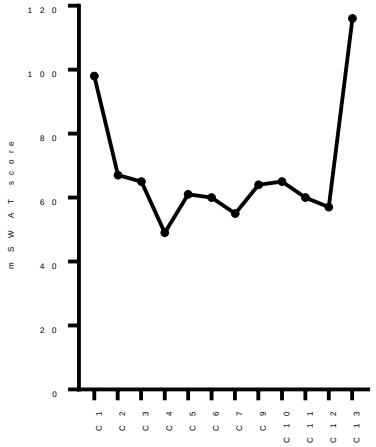
Patient 3 (2102): Cutaneous TCL – Sézary-Syndrome (SS)

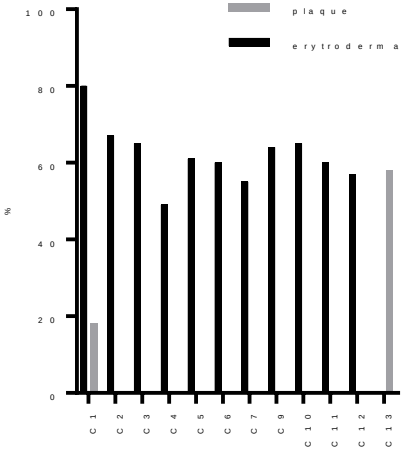
Patient	55 year old female with CTCL-SS, diagnosed 2010
Tumor	Skin T4, Nx, M0, B2 (stage IV)
Doses	12, 1 mg/kg q3w, off study due to PD

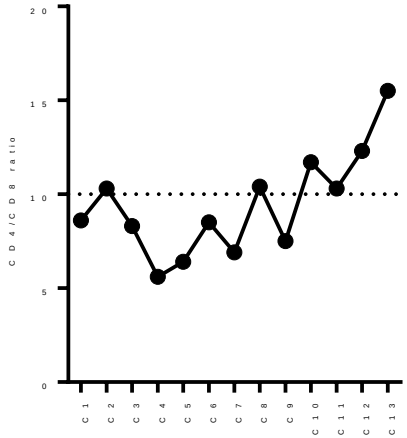
Treatments and best response



mSWAT score, total body lesions and CD4/CD8 ratio in blood







- PR in skin
- SD in nodes and blood
- Best response PR

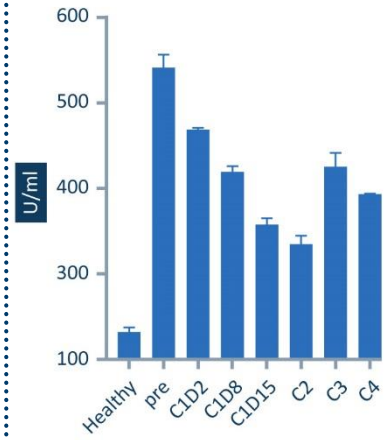
- Cutaneous tumor lesions decrease in surface area; 50% reduction of mSWAT and a partial response (PR)
- Cutaneous tumor lesions improve from plaques to erythroderma, but increased plaque at PD

Source: argenx data –patient anecdotes - uncleaned

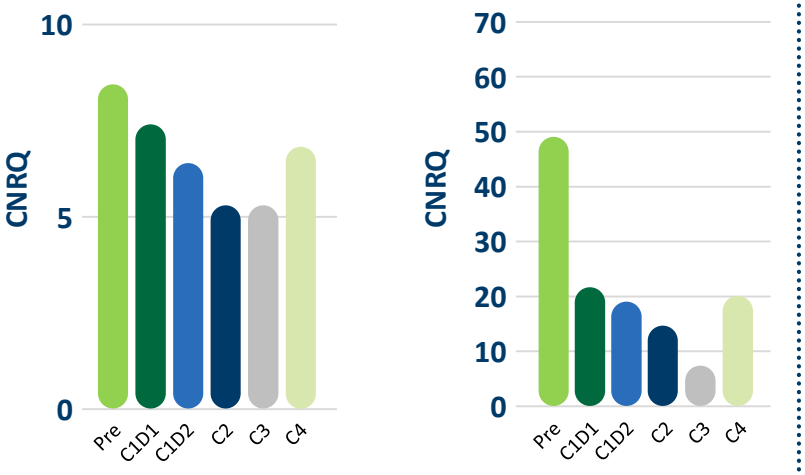
Decreased sCD27 and Sézary clone in the blood

Patient 3: Cutaneous TCL – Sézary-Syndrome (SS)

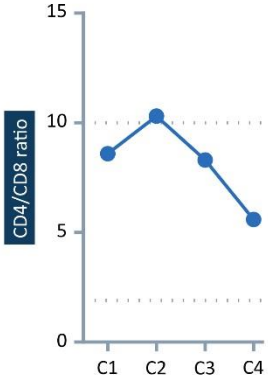
sCD27 levels in serum



RNA CCR4 & RNA TWIST1



CD4/CD8 cells in blood



- Decrease in sCD27 first 2 cycles; then stabilization
- Sézary clone levels decrease as shown by CD4/CD8 ratio and CCR4 & TWIST1 levels (RNA)

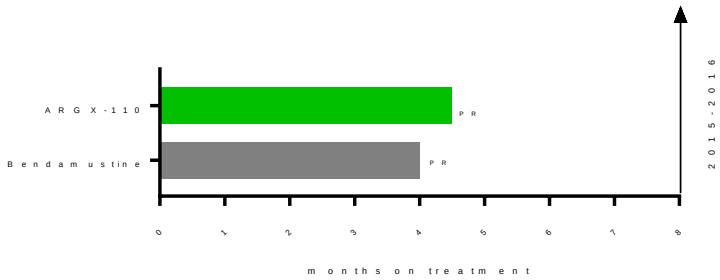
Source: argenx data –patient anecdotes - uncleaned

Partial respons: improvement subcutaneous lesions

Patient 4 (2203): Cutaneous TCL – Panniculitis like

Patient	84-year old female, diagnosed June 2015
Tumor	Skin T3, Nodal N0, Visceral M0, Blood B0
Nr doses	6, ongoing

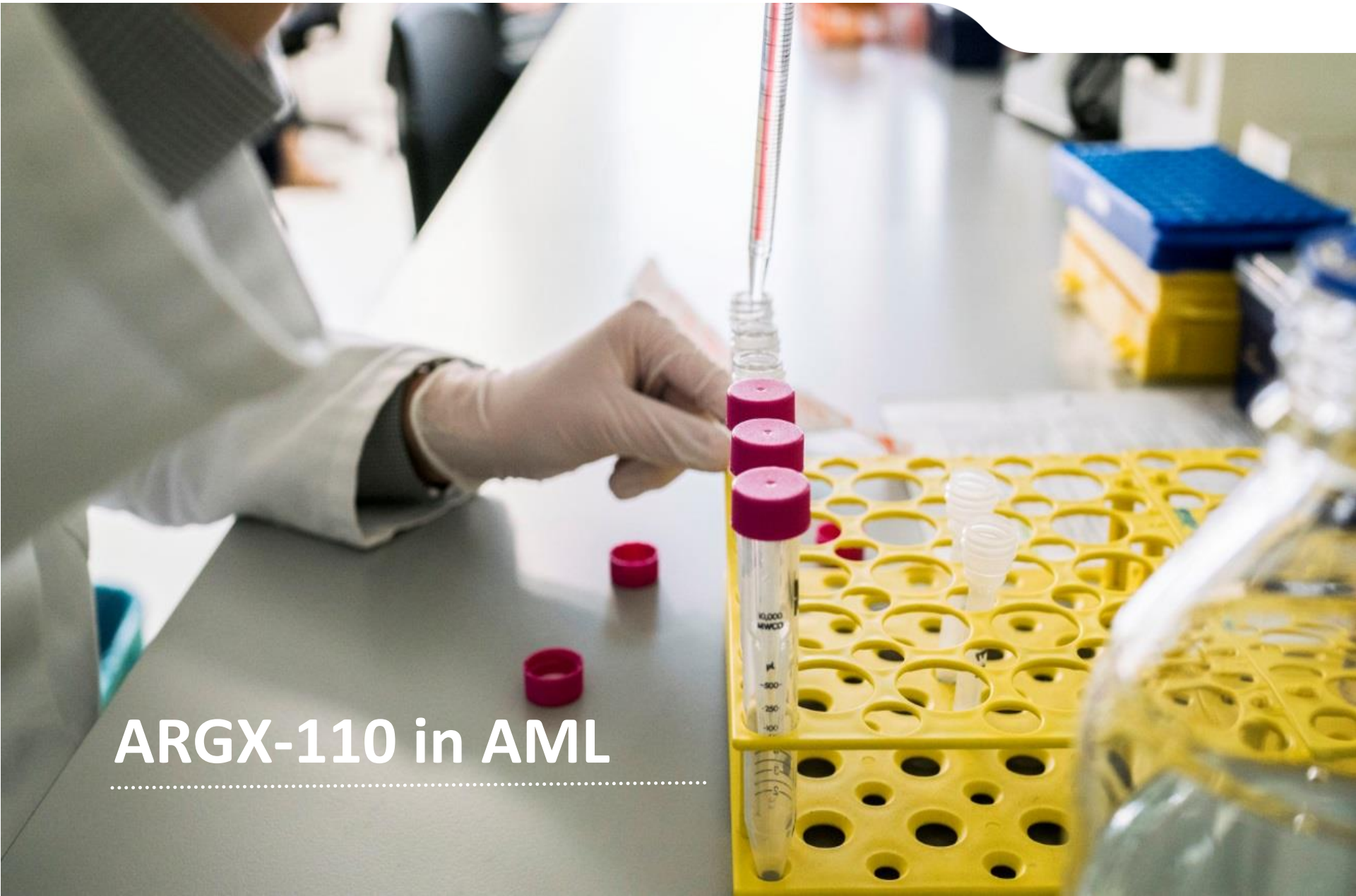
Treatments and best response



Overview

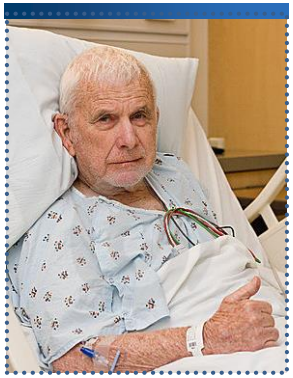
	Prescreen	C1	C2	C3	C4	C5	C6	C7
FDG-PET global response in skin			C2D20 SD		C4D1 SD		C6D1 PR	
IHC CD70%	Fresh, cutaneous 51-75%, 3+					No tumor cells detected		

Source: argenx data –patient anecdotes - uncleaned



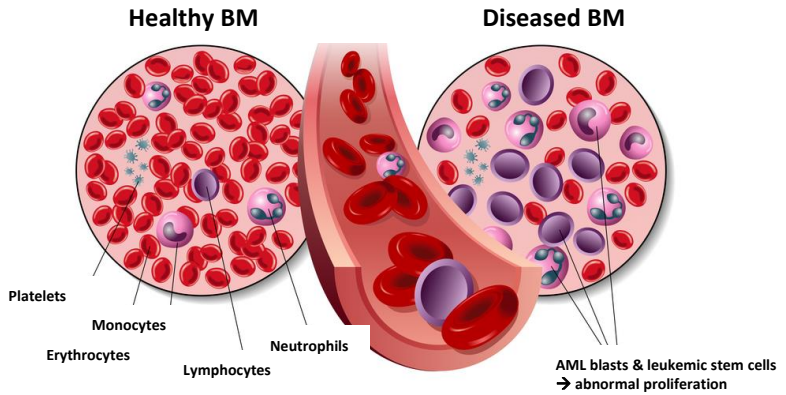
ARGX-110 in AML

Acute Myeloid Leukemia: Fact sheet



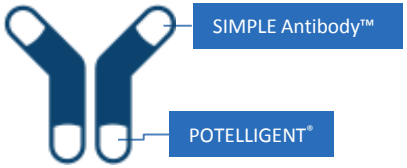
Fatigue, shortness of breath, easy bruising bleeding, progresses rapidly, fatal if left untreated

- Rare disease
- Incidence (US): 19,950 new cases/year
- Disease of the elderly
- Worst 5Y survival rate of heme malignancies (cfr. acute)



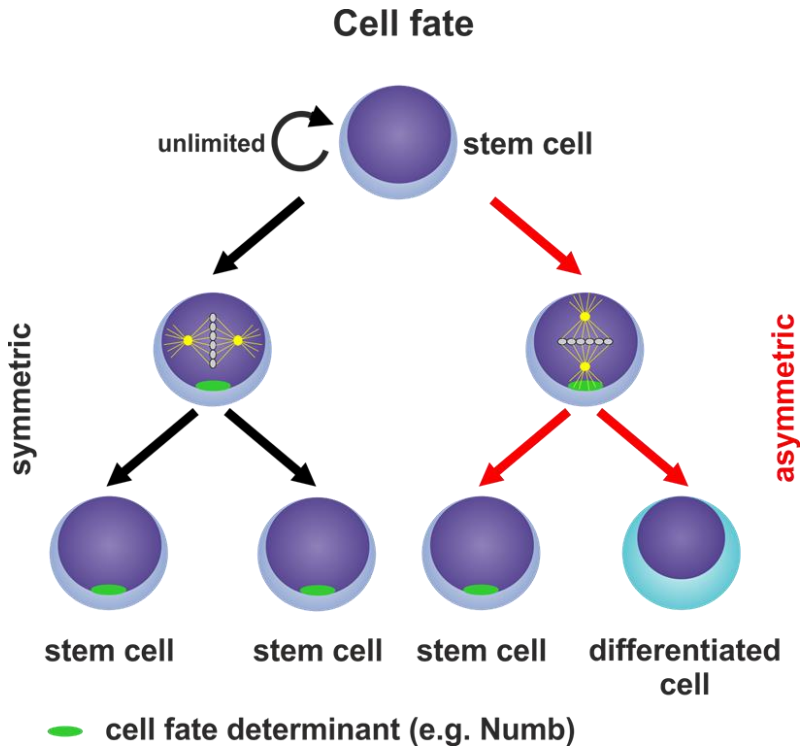
Treatment options

- Younger patients
  - Standard chemotherapy: 7+3 regimen/transplant
  - Clinical response: short-lived, survival: 8-10 months
- Older patients
  - Older patients (>65) unfit for transplant
  - Palliative treatment with hypomethylating agents (ao Azacitidine – median survival: 7-10 months)



- Potential for ARGX-110 in AML
  - CD70/CD27 highly overexpressed in newly diagnosed AML patients
  - CD70/CD27 signaling correlates with poor prognosis
  - CD70 selectively overexpressed on leukemic and not hematopoetic stem cells → selective tumor targeting by ARGX-110
  - Azacytidine upregulates CD70 expression → combination with ARGX-110: start PhaseI/II trial

AML: Leukemic stem cells responsible for disease relapse

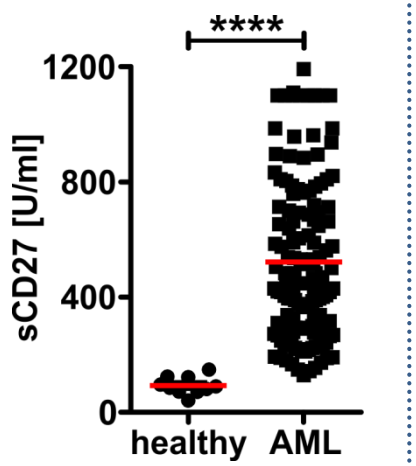


- Accumulation of blasts in bone marrow and blood → drop in red blood cells, platelets, and normal white blood cells
- Leukemic stem cells (LSCs = AML stem/progenitor cells) are responsible for disease relapse

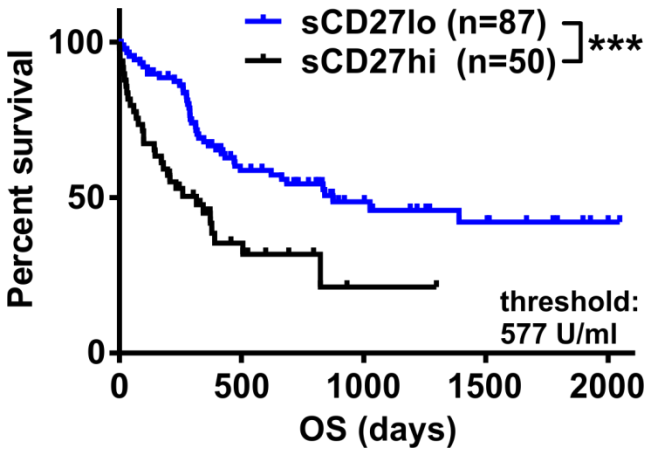
ARGX-110: Rationale in AML

Indication for CD70/CD27 signaling in AML patients

sCD27 serum levels



sCD27 serum levels → poor prognosis



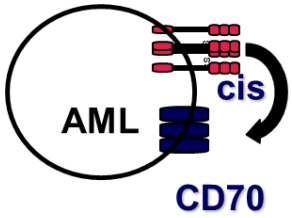
parameter	HR (95% CI)	p-value
sCD27	2.17 (1.34-3.50)	0.0016
risk group	1.69 (1.29-2.38)	0.0024
age	1.03 (1.01-1.05)	0.0050
BM blast %	0.99 (0.98-1.00)	0.1259
blood blast %	1.00 (0.99-1.01)	0.9329
blood leukocyte #	1.00 (0.99-1.01)	0.6558

- sCD27 serum levels:
  - biomarker for active CD70/CD27 signaling in vivo
  - increased in serum of AML patients
  - independent negative prognostic marker across entire patient population

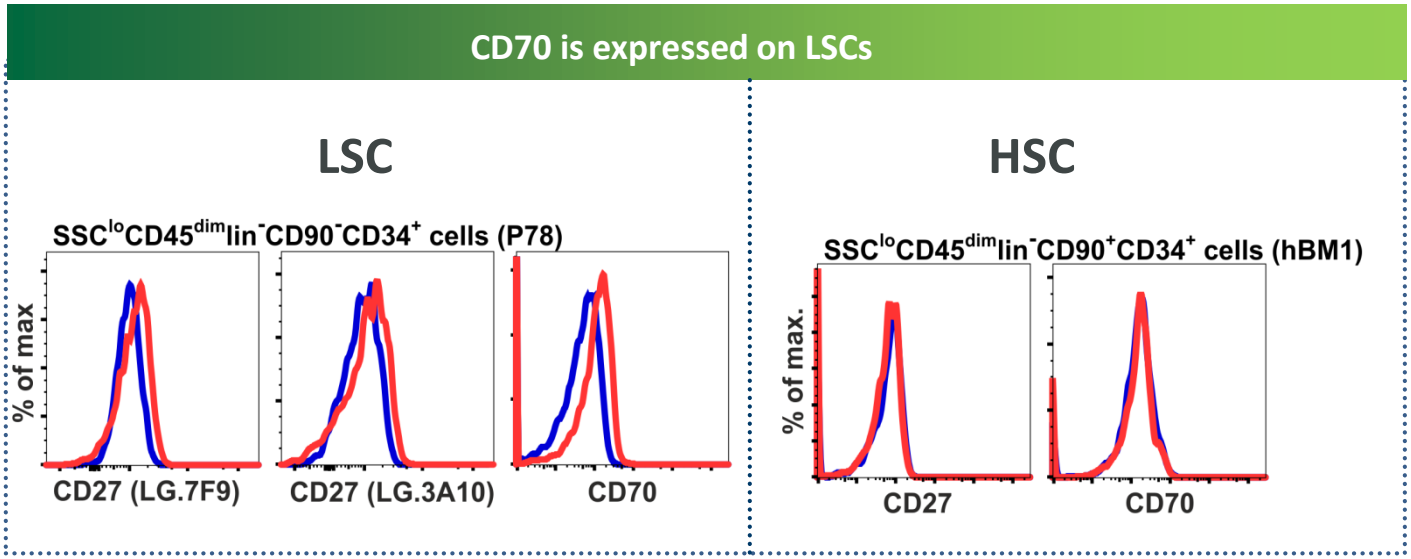
Riether et al, Journal of Experimental Medicine, 2016

ARGX-110: Rationale in AML

CD70/CD27 biology highly involved in newly diagnosed AML



CD70 is expressed on LSCs

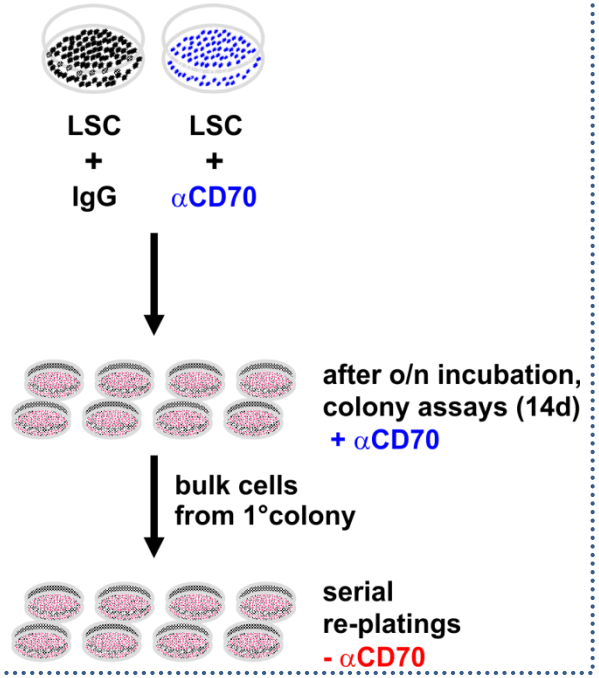


- CD70/CD27 selectively overexpressed on LSCs and not on hematopoietic stem cells (HSC)
- CD70 expressed on ~100% of AML blasts, majority of malignant cells are CD70/CD27 double-positive
- ARGX-110: selective targeting of LSCs

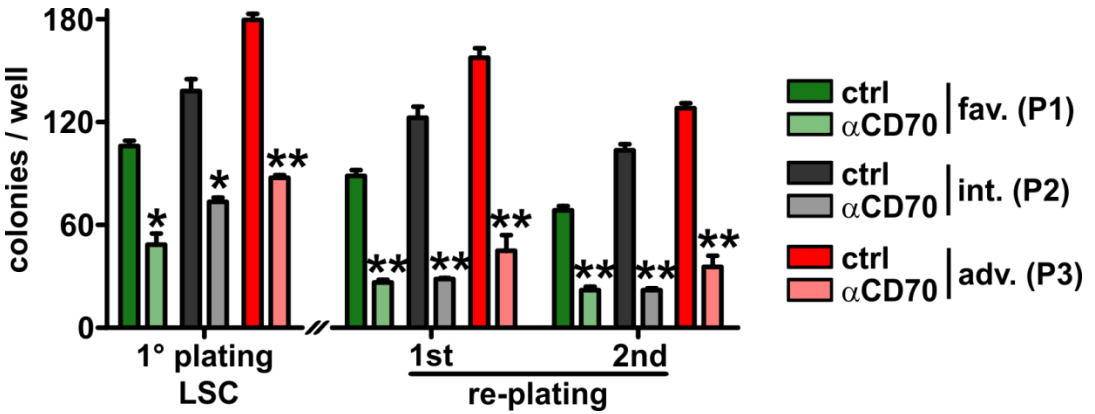
Riether et al, Journal of Experimental Medicine, 2016

ARGX-110: Leukemic stem cell function ex vivo

Long-term effects *ex vivo*



αCD70 reduces LSC colony formation

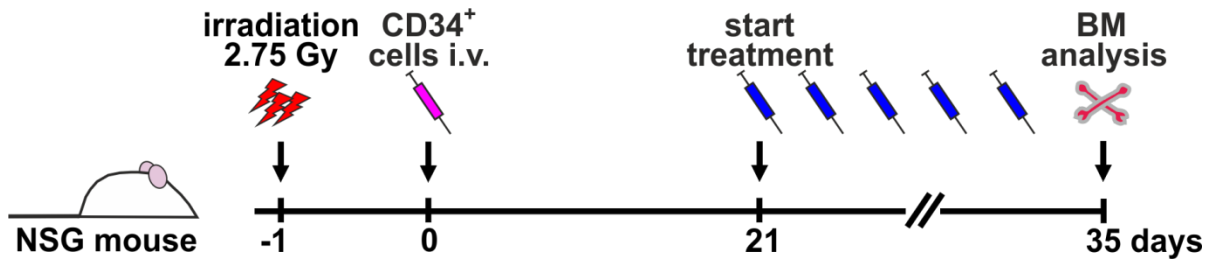


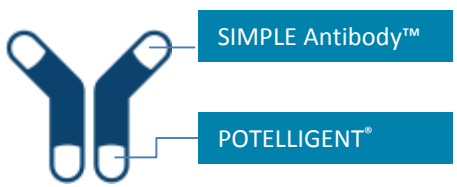
- αCD70 Ab reduces colony formation of LSC
- αCD70 Ab reduces LSC numbers as determined in serial re-plating experiments

Riether et al, Journal of Experimental Medicine, 2016

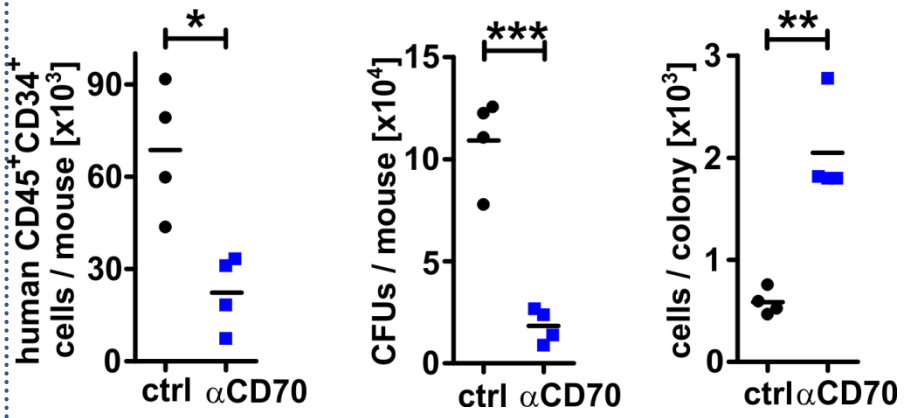
ARGX-110: Periodic treatment

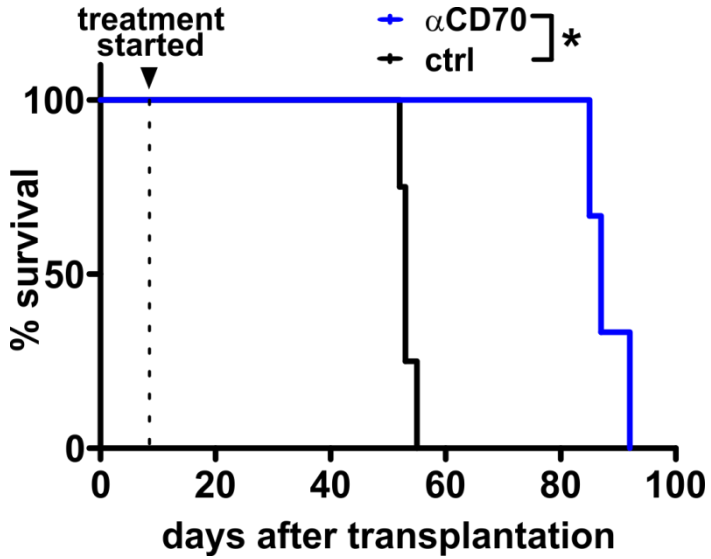
Reduction of LSCs cell numbers and function





αCD70 mAb treatment reduces LSCs cell function *in vivo* and prolongs survival

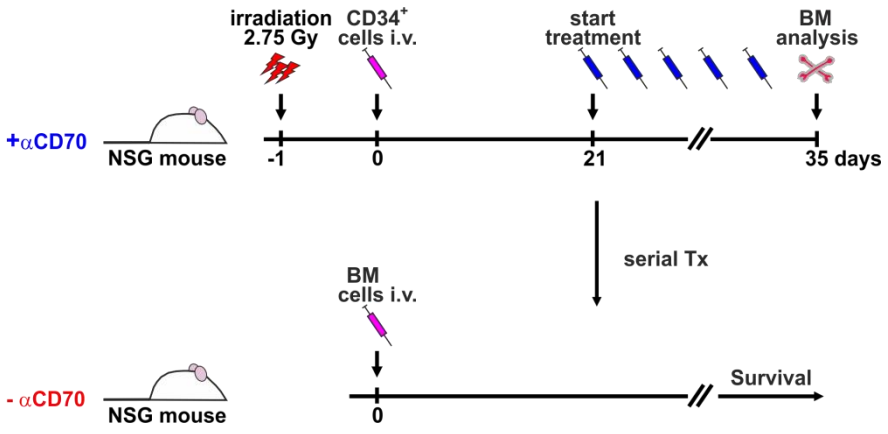




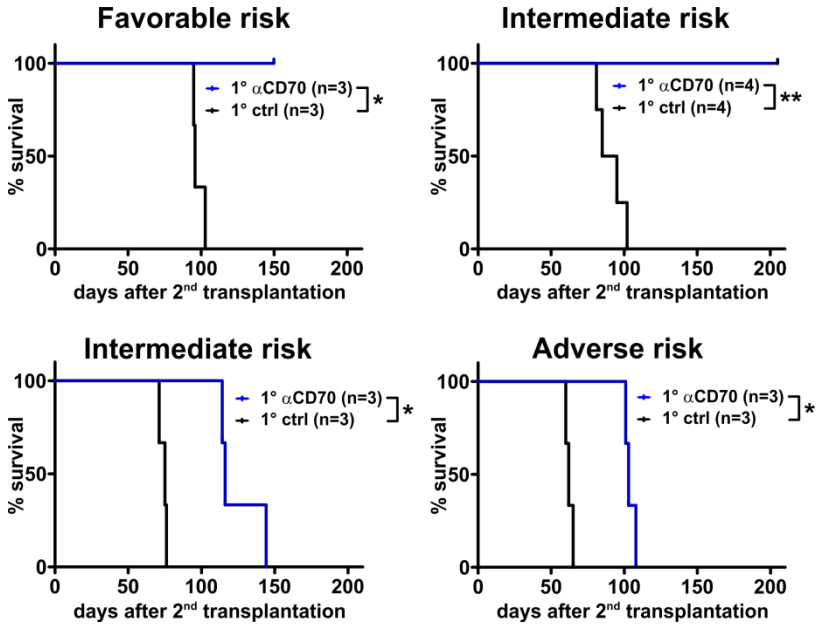
Riether et al, Journal of Experimental Medicine, 2016

ARGX-110: Periodic treatment

Long-term effects *in vivo*



BM of αCD70-treated contain fewer cells that transmit the disease (LSCs) in vivo

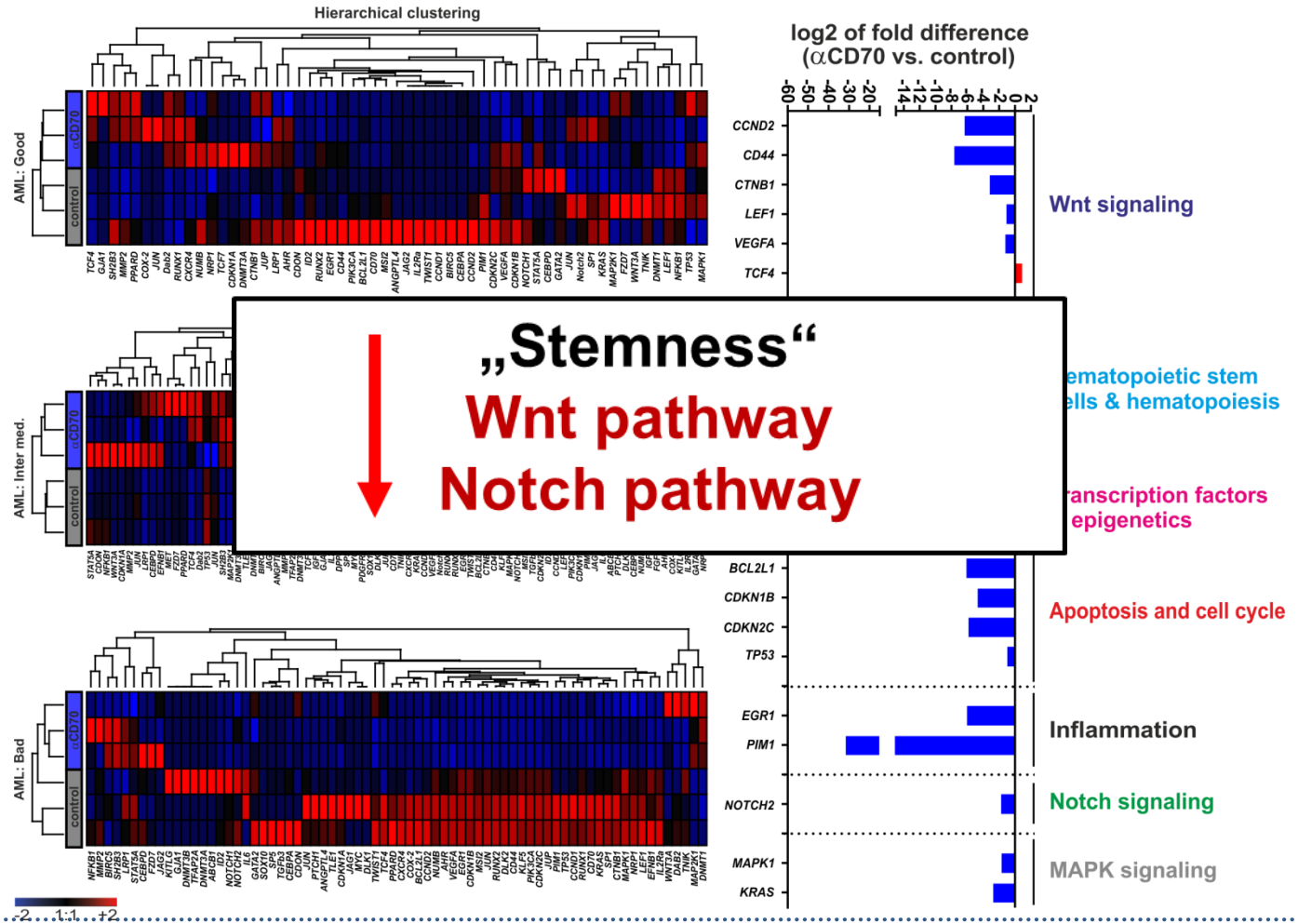


- Increased survival after secondary transplantation of AML BM cells from primary recipients transiently treated with αCD70 Ab
- Increased survival observed for AML blasts taken from all 3 AML risk categories

Riether et al, Journal of Experimental Medicine, 2016

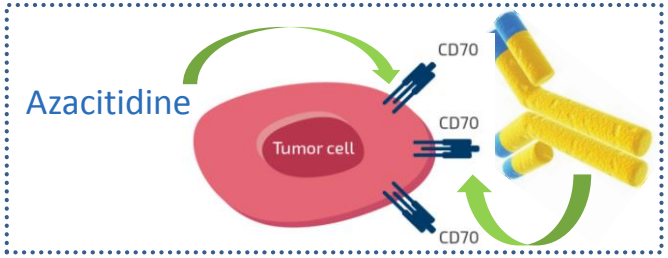
Blocking CD70/CD27 signaling

Reduction in stem cell characteristics



Riether et al, Journal of Experimental Medicine, 2016

Phase I/II Combo ARGX-110 & Azacitidine: trial design



Phase I – Dose escalation

Safety and tolerability

Vidaza = 75 mg/m2 (standard of care)

10mg/kg

N = 3+3

3mg/kg

N = 3+3

1mg/kg

N = 3+3

N = up to 18

Phase II – Proof of concept

Efficacy

selected ARGX-110 dose

N = 15

selected ARGX-110 dose

N = 9 – (3-6 from Ph I)

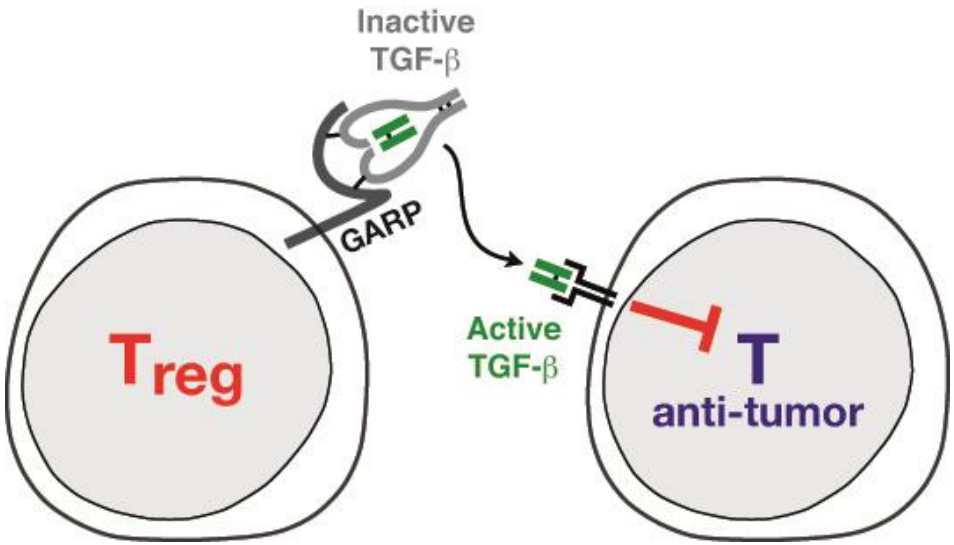
N = up to 24

- Population: untreated AML & high risk of myelodysplastic syndrome, eligible for AZA
- Design: open-label, non-controlled, non-randomized

ARGX-115

Cancer Immunotherapy

ARGX-115: Towards a next generation Yervoy

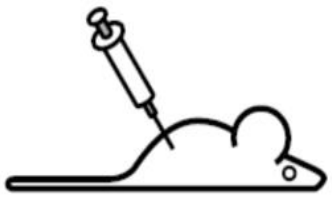


- GARP upregulated specifically on surface of Tregs only
- GARP presents and activates latent TGF-ß1, activating Tregs and suppressing Teff cells
- SIMPLE Antibody™ hitting unique, patented epitope on GARP
- GARP blockade sufficient for MoA – no Treg depletion
- Graft-versus-host-disease model delivered convincing PoC

Cuende et al., 2015, Science Translational Medicine

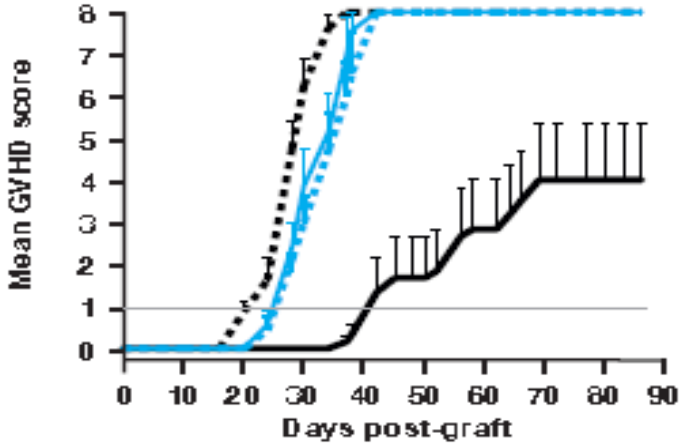
ARGX-115: Towards a next generation Yervoy

In vivo efficacy of anti-GARP-TGFβ SIMPLE Antibody™ in GVHD Model



NSG mice injected with:

hPBMC →
+/- hTregs →
+/- anti GARP →



- PBMCs
- PBMCs + Tregs
- PBMCs + Tregs + LHG-10.6
- PBMCs + Tregs + LHG-10.6$_{N297Q}$

Cuende et al., 2015, Science Translational Medicine

AbbVie option deal

Key elements

Financial terms

- $ 40MM upfront
- Preclinical milestones 2x $10MM
- Up to $ 625MM development, regulatory and commercial milestones
- Tiered, up to double-digit royalty payments on net product sales

Deal highlights

argenx

- Responsible for delivering IND data package
- GARP-based research programs creating further product opportunities
- Retains rights to combine ARGX-115 with own pipeline programs
- Co-promotion rights in EU/Swiss Economic Area

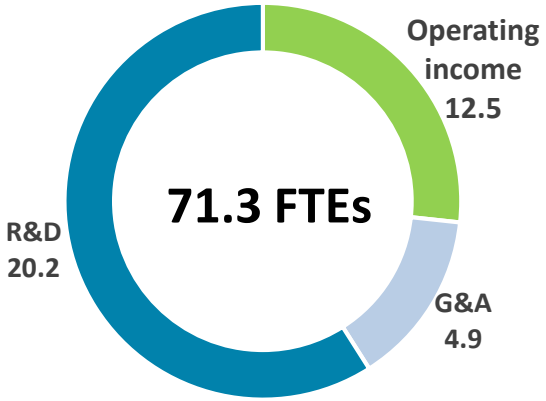
abbvie

- Option to exclusive development and commercialization license
- Further GARP-based research funding once first preclinical milestone met
- Right to license additional therapeutic programs resulting from this research - additional milestone and royalty payments on resulting products

Financials

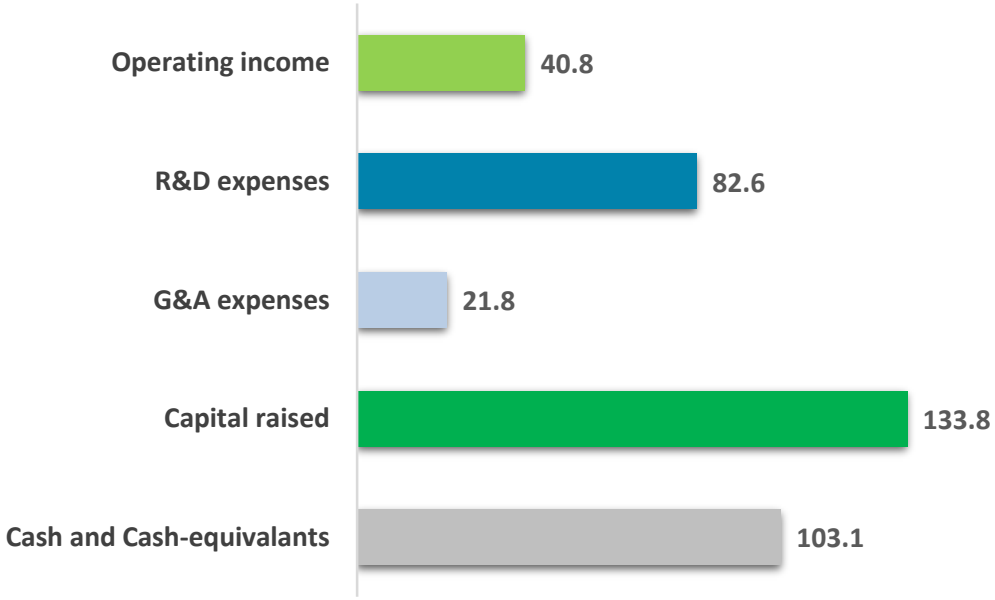
Well capitalized to execute strategic plan

Operating income & expenses
3Q16 (MEUR)

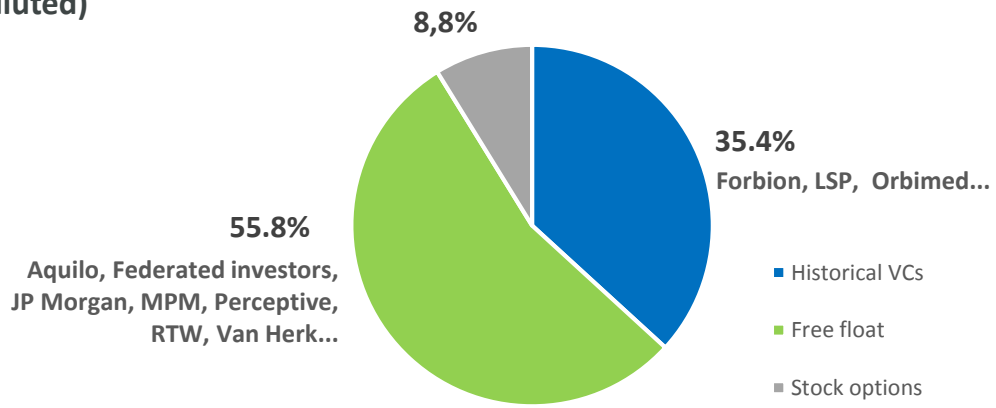


Operating income, expenses & capital raised since inception (*)
3Q16 (MEUR)

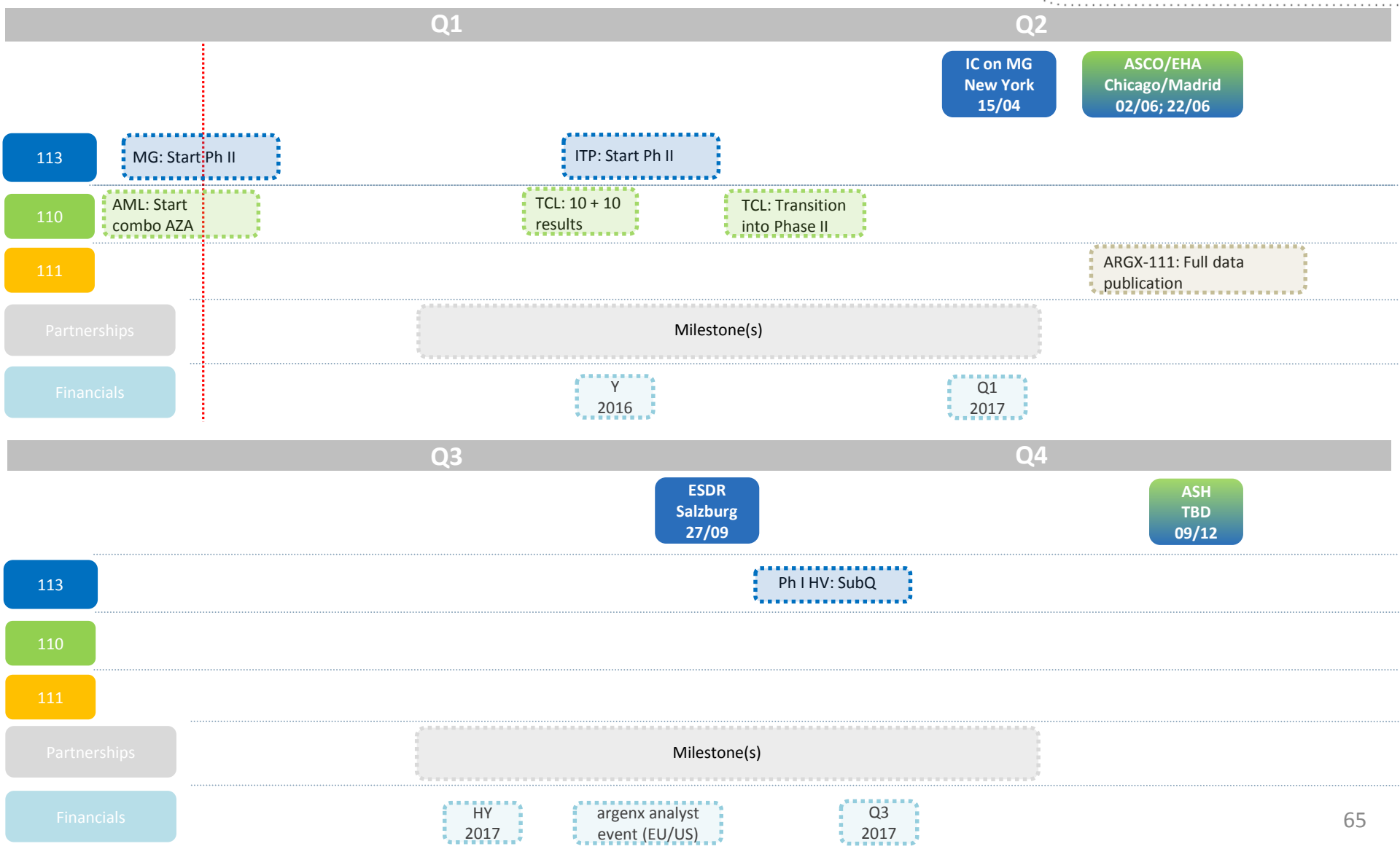
(*) not including deferred revenue and accruals

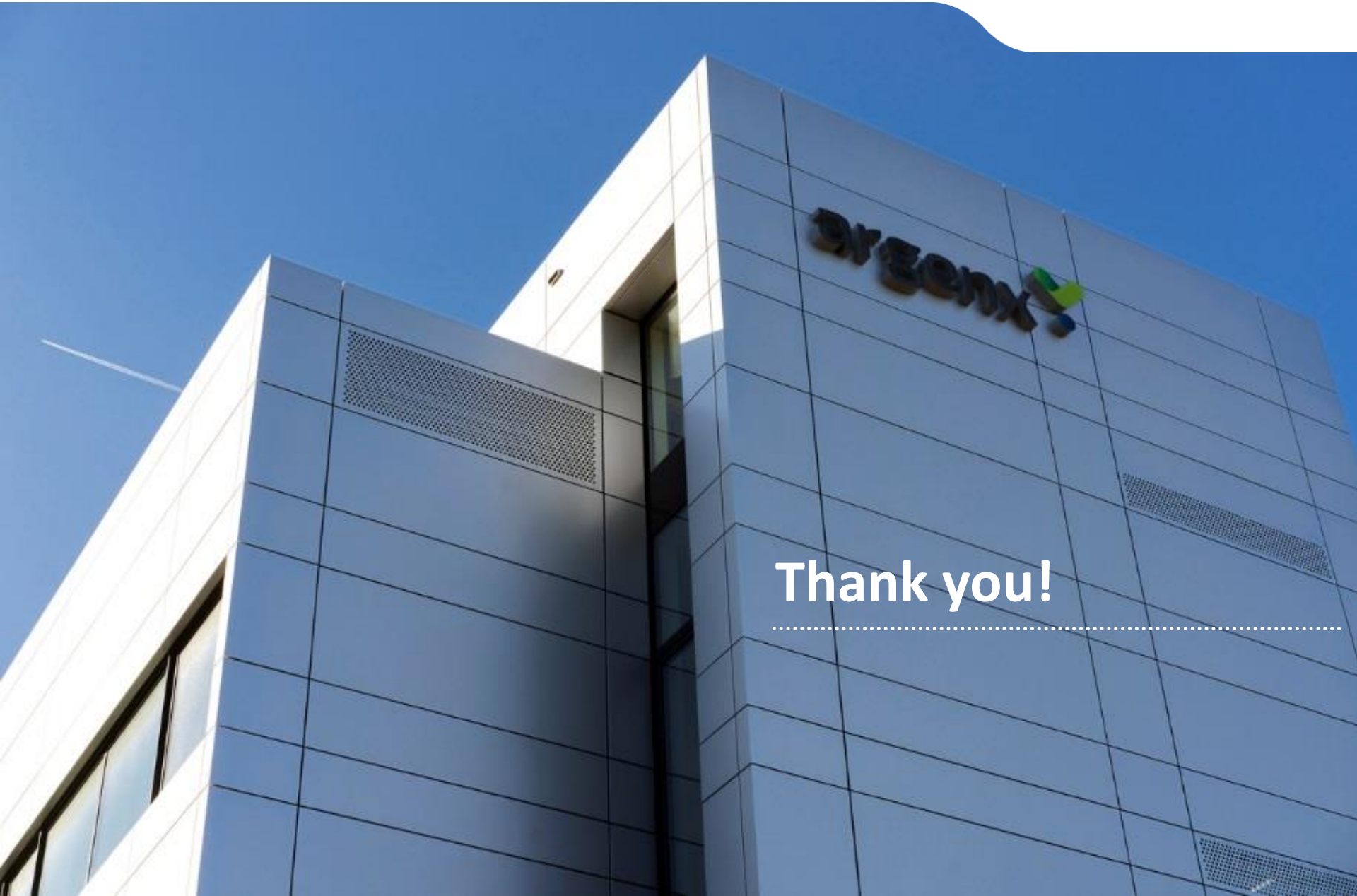


Shareholder structure (fully diluted)
Dec 2016



Communications plan 2017





Thank you!