TAKING DEVASTATING DISEASES – AND DEVASTATING THEM.

June 24, 2023

We're going there.

Rallybio

ISTH Investor Event

Forward-Looking Statements

This presentation contains forward-looking statements that are based on management's beliefs and assumptions and on information currently available to management. All statements other than statements of historical facts contained in this presentation are forward-looking statements. In some cases, you can identify forward-looking statements by terms such as "may," "will," "should," "expect," "plan," "anticipate," "could," "intend," "target,", "seek," "goal," "project," "contemplate," "believe," "estimate," "predict," "potential" or "continue" or the negative of these terms or other similar expressions, although not all forward-looking statements contain these words. Forward-looking statements include, but are not limited to, statements concerning: the initiation, timing, progress, results, and cost of our research and development programs, and our current and future preclinical and clinical studies, including statements regarding our clinical development plan for RLYB212; the timing of initiation and completion of our clinical trials for RLYB212 and RLBY116, and the natural history study for our FNAIT prevention program, and related preparatory work, and the period during which the results of the trials will become available; the success, cost and timing of our clinical development of our product candidates, including RLYB212 and RLYB116; the results from the Phase 1 study of RLYB116 and the multiple ascending dose study for RLYB116; the proof-of-concept data from the Phase 1b study of RLYB212 for the prevention of FNAIT; the potential clinical effects and benefits of RLYB212; our ability to initiate, recruit and enroll patients in and conduct our clinical trials at the pace that we project; our ability to obtain and maintain regulatory approval of our product candidates, and any related restrictions, limitations or warnings in the label of any of our product candidates, if approved; our ability to compete with companies currently marketing or engaged in the development of treatments for diseases that our product candidates are designed to target, including FNAIT; our reliance on third parties to conduct our clinical trials; our reliance on third parties to manufacture drug substance for use in our clinical trials; the size and growth potential of the markets for RLYB212 and RLYB116 and any of our current product candidates or other product candidates we may identify and pursue, and our ability to serve those markets; our ability to successfully develop and validate screening tests for FNAIT, and whether such tests will be accepted in routine prenatal guidelines; our ability to expand our pipeline through collaborations, partnerships and other transactions with third parties; our ability to retain and recruit key personnel; our expectations regarding government and third-party payor coverage and reimbursement; our estimates of our expenses, ongoing losses, capital requirements and our needs for or ability to obtain additional financing; the potential benefits of strategic collaboration agreements, our ability to enter into strategic collaborations or arrangements, including potential business development opportunities and potential licensing partnerships; and our financial performance. The forward-looking statements in this presentation are only predictions and are based largely on management's current expectations and projections about future events and financial trends that we believe may affect our business, financial condition and results of operations. These forward-looking statements speak only as of the date of this presentation and are subject to a number of known and unknown risks, uncertainties and assumptions, including but not limited to, our ability to successfully initiate and conduct our planned clinical trials and complete such clinical trials and obtain results on our expected timelines, or at all, whether our cash resources will be sufficient to fund our operating expenses and capital expenditure requirements and whether we will be successful raising additional capital, competition from other biotechnology and pharmaceutical companies, and those risks and uncertainties described in our filings with the Securities and Exchange Commission (the "SEC"), including under the heading "Risk Factors" in our Form 10-Q for the quarter ending March 31, 2023, and any subsequent filings with the SEC. Because forward-looking statements are inherently subject to risks and uncertainties, some of which cannot be predicted or quantified and some of which are beyond our control, you should not rely on these forward-looking statements as guarantees of future events. The events and circumstances reflected in our forward-looking statements may not be achieved or occur and actual future results, levels of activity, performance and events and circumstances could differ materially from those projected in the forward-looking statements. Moreover, we operate in an evolving environment. New risks and uncertainties may emerge from time to time, and it is not possible for management to predict all risks and uncertainties. Except as required by applicable law, we are not obligated to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise.

Agenda

1. Welcome – Overview of Rallybio
2. FNAIT / RLYB212 Overview
3. RLYB212 PoC Data Presentation by Dr. Geisen / Q&A
4. Clinical Development Update for RLYB212 / Q&A
5. Closing

Rallybio's Mission: Profoundly Transform the Lives of Patients

Advancing a diversified portfolio

to address compelling unmet needs, including in maternal-fetal health, complement dysregulation, hematology and metabolic diseases

Proven team of innovators

charting new paths based on industry-leading science and expertise in rare diseases

Strong financial position

$150.4 million in cash, cash equivalents and marketable securities as of March 31, 2023

Successful business development platform

global relationships result in a robust portfolio of novel programs

Experienced Team in Rare Diseases R&D

Martin Mackay, Ph.D.
Co-Founder and Chief Executive Officer
Alexion, AstraZeneca, Pfizer

Jonathan Lieber
Chief Financial Officer
AGTC, Histogenics, Danforth Advisors

Róisín Armstrong, Ph.D.
Head of FNAIT Program
Alexion, Pfizer

Ann Houston, M.A., PMP
Head of External Research & Innovation
Alexion, Bristol-Myers Squibb, Pfizer

Douglas Sheridan, Ph.D.
Head of Non-Clinical Development
Alexion

Rachael Alford, Ph.D.
Head of CMC and Integrated Operations
Alexion

Jackie Schumacher
Head of Regulatory and Quality
Lyndra Therapeutics, Pfizer

Steve Uden, M.D.
Co-Founder, President and Chief Operating Officer
Alexion, Wyeth, Novartis, Pfizer

Steve Ryder, M.D.
Chief Medical Officer
Alexion, Astellas, Pfizer

Eric Watsky, M.D.
Head of RLYB116 Program
Alexion, Pfizer, NIMH

Kiran Patki, M.D., M.Sc.
Head of RLYB114 and RLYB331 Programs
Gemini, Aeglea, Alexion

Amanda Hayward, Ph.D.
Global Head of Business Development
Connecticut Innovations, Baxalta Ventures

Laura A. Ekas, Ph.D.
Head of Corporate and Commercial Strategy
Alexion, Insight Strategy, Canaccord

Derek Brown, MBA
Global Commercial Development Lead
Chiasma, Alexion, Boehringer Ingelheim

Rapidly Advancing Our Diversified Portfolio

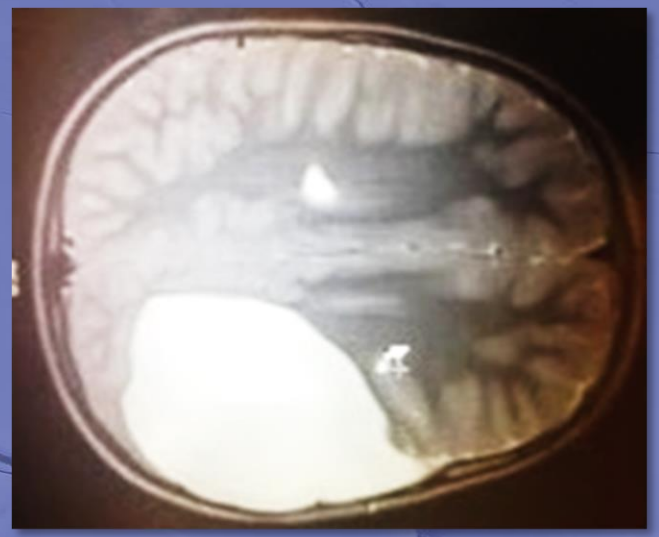
Therapeutic Area	Program	Molecule	Approach	Development Rights
Maternal Fetal Blood Disorders	Prevention of FNAIT	**RLYB212**	Anti-HPA-1a Monoclonal Antibody	Rallybio
Complement Dysregulation	Rare Diseases*	**RLYB116**	C5 Inhibitor: Affibody®-ABD Fusion	Rallybio
	Ophthalmology	**RLYB114****	C5 Inhibitor: Pegylated Affibody®	Rallybio
Hematology	Severe Anemia	**RLYB331**	Matriptase-2 Inhibitor; Monoclonal Antibody	Rallybio
Metabolic Disorders	HPP	**ENPP1 program**	ENPP1 Small Molecule Inhibitor	Rallybio, Exscientia
	Undisclosed	**Undisclosed**	Undisclosed	Rallybio, AbCellera

FNAIT: Fetal and neonatal alloimmune thrombocytopenia; **HPA-1a:** Human platelet antigen-1a; **C5:** Complement component 5; **ABD:** Albumin-binding domain; **HPP:** Hypophosphatasia; **ENPP1:** Ectonucleotide pyrophosphatase/phosphodiesterase 1
*Disease areas under consideration: hematology, including disorders such as paroxysmal nocturnal hemoglobinuria (PNH), neurology, including disorders such as generalized myasthenia gravis (gMG), and severe dermatological indications ; ** Rallybio and EyePoint Pharmaceuticals are conducting an evaluation to assess the viability of using EyePoint's delivery technology with Rallybio's complement inhibitor; following the evaluation, the parties will determine whether to advance the collaboration, and EyePoint would assume development rights

RLYB212

Potential Preventative Treatment for Fetal and Neonatal Alloimmune Thrombocytopenia (FNAIT)

RLYB212: Potential to Prevent a Devastating Disease



Fetal and Neonatal Alloimmune Thrombocytopenia (FNAIT) results from platelet antigen (HPA-1a) mismatch between an expectant mother and her fetus. This can lead to alloimmunization (maternal immune system attacks fetal platelets).

No approved therapy exists for the prevention or treatment of FNAIT, which is associated with intracranial hemorrhage (ICH) and:

- Severe, life-long neurological disability
- Miscarriage or stillbirth
- Loss of the newborn

RLYB212 is a novel candidate designed to prevent HPA-1a alloimmunization and to eliminate FNAIT.

Significant Number of Pregnancies at Risk for FNAIT Each Year

>22,000*

Are at higher risk for FNAIT each year where there is an HPA-1 mismatched fetus and the expectant mother is:

- HPA-1a negative
- HLA-DRB3*01:01 positive
- HPA-1a antibody negative

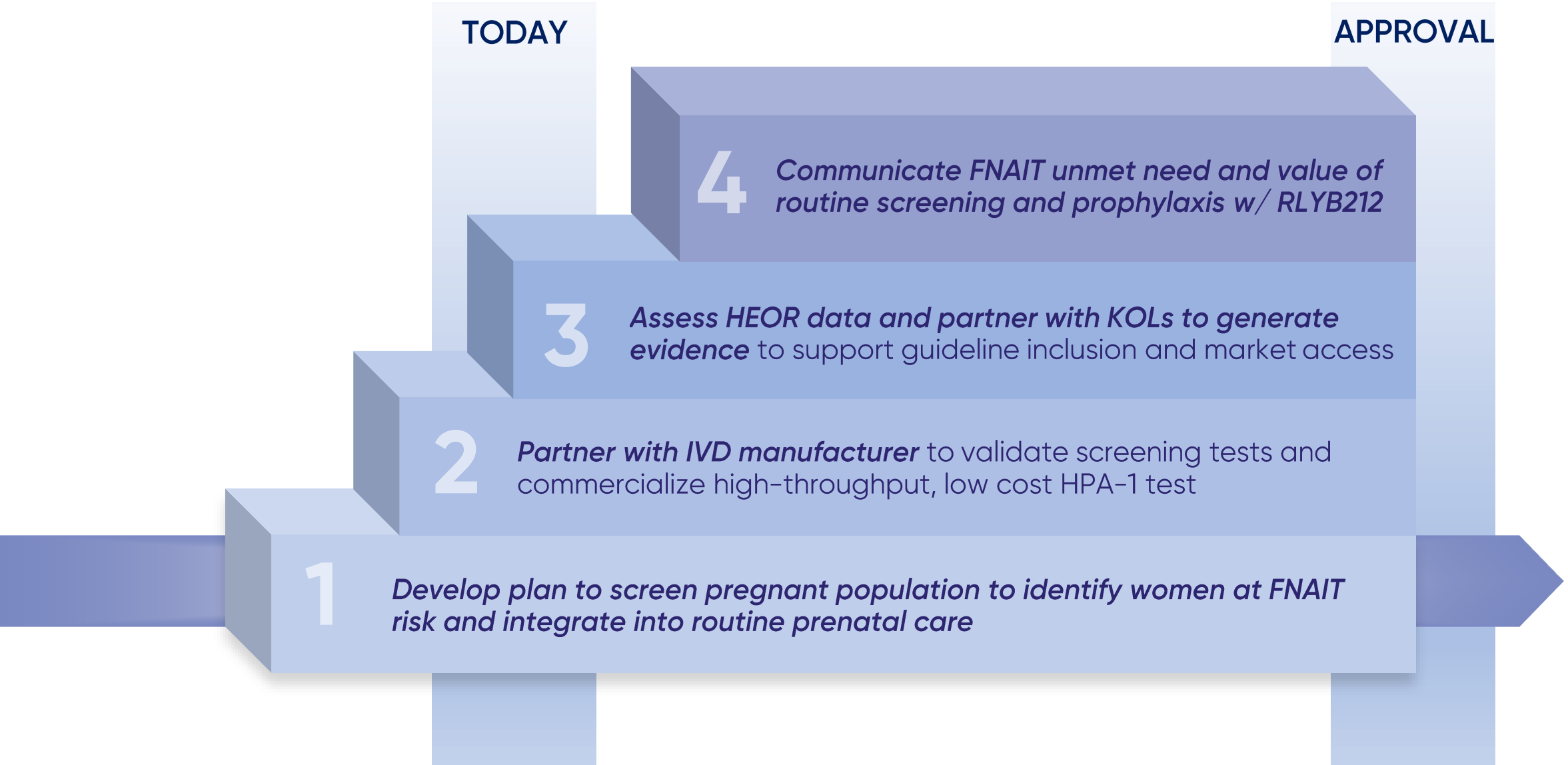
(based on 8M live births each year in the US, Canada, UK, major EU Countries, and Australia)

US and European OB/GYNs and maternal-fetal specialists have**:

- High awareness of catastrophic impact of FNAIT and need for a preventative therapy
- Favorable response to RLYB212 target profile

Estimate is based on a ~2% incidence of HPA-1a negative status in the Caucasian population, where the prevalence has been well-characterized. Data from ongoing Natural History Study will inform incidence in non-Caucasians.
*Source: NCHS National Vital Statistics Report Volume 68, Number 13, November 30, 2019, Births: Final Data for 2018; World Bank Population Data (2018); Kjeldsen-Kragh, et al Blood 2007; Hardy-Weinberg estimate; Kjeldsen-Kragh et al Blood 110, 833-839 (2007)
** Based on 2022 Rallybio market research study with 30 US and European HCPs and payers

Success Factors to Support Commercialization of RLYB212



Screening Tests Commercially Available; Can be Validated in FNAIT and Integrated into Routine Prenatal Care

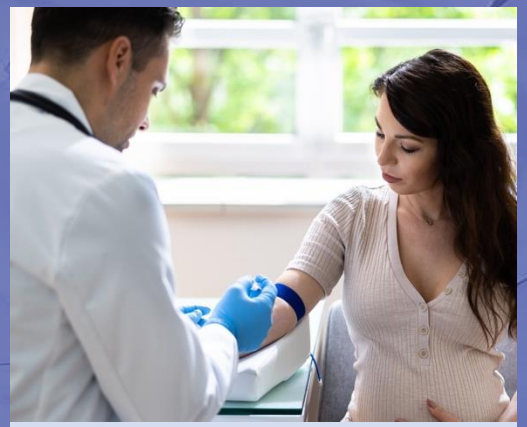
Existing Routine Blood Tests*

FNAIT Risk Test To Be Added

FIRST TRIMESTER

- ☑ Blood type
- ☑ Rh factor
- ☑ CBC: H&H, MCV
- ☑ Platelet Count
- ☑ Hepatitis B
- ☑ Blood glucose
- ☑ Syphilis, STDs, HIV
- ☑ Genetic & infectious diseases
- ☑ PAPP-A, hCG
- ☑ Down Syndrome, trisomy 18
- ☑ Maternal HPA-1 typing

FNAIT Screening



SCREENING PROCESS

- Low volume sample added to standard maternal blood typing panel
- Analyzed at reference labs and hospitals on existing equipment

If **HPA-1a negative**
(~2%** of pregnancies)

Additional testing to evaluate **HLA-DRB3*01:01 status**. If positive, referral to specialist as high-risk pregnancy***

* Summarized from ACOG Guidelines for Perinatal Care
** Estimate is based on a ~2% incidence of HPA-1a negative status in the Caucasian population
*** For pregnancies identified at higher FNAIT risk, physicians may conduct additional follow-up testing, e.g., maternal anti-HPA-1a antibody status, fetal HPA-1a genotype

Activities Ongoing to Support Inclusion in Guidelines and Market Access

Criteria for Payer and Guideline Evaluations

- **Prevalence & Impact Data**
- **Preventive Therapeutic**
- **Validated Screen Test**
- **Cost Effectiveness Data**

Action Plan

Sci Advisors & Patient Advocacy	• Collaborating with top OB/GYN & MFM experts on development program • Building relationships with patient advocacy organizations
Natural History & Phase 2/3 Data	• Characterizing ethnically diverse populations at higher risk of FNAIT in Natural History Study who would be eligible for prophylactic treatment • Planning global registration program to establish the effectiveness of screening and RLYB212
Screen Testing Readiness	• Identifying global IVD partner to validate existing tests in Phase 2/3 and to provide commercial low cost HPA-1 screen test
Cost/Benefit Payer Insight	• Engaging with payers to confirm data requirements for market access
Publications & Guidelines Review	• Partnering with FNAIT thought leaders to generate and disseminate data to support guideline and market access decisions

With >22,000 Women at Higher Risk for FNAIT Annually, RLYB212 Represents a $1B+ Market Opportunity

Drivers of Commercial Opportunity

Unmet Medical Need

- >22,000 women at higher risk of potentially devastating outcomes, including ICH in ~1/10 FNAIT affected pregnancies
- HCPs and payers understand limitations of current approaches and value prophylactic approach

Existing Care is Resource Intensive

- Lifetime medical costs for Cerebral Palsy estimated to be $921,000*
- Extended and costly NICU stays
- IVIg treatment costs for alloimmunized mothers range from $100k-$300k in US (per pregnancy)

Readily Identifiable Population

- Screening tests already commercially available
- Tests can be seamlessly integrated into routine prenatal care
- Payers, HCPs, and pregnant mothers are highly motivated to deliver healthy babies

Broad and Rapid Utilization

- Reimbursement currently available for existing HPA-1a screen tests which will facilitate screening uptake at launch
- Current analysis indicates modest budget impact
- Guideline adoption will drive rapid and complete adoption of screening and treatment

*Source: CDC MMWR Weekly January 30, 2004 53(03);57-59, Economic Costs Associated with Mental Retardation, Cerebral Palsy, Hearing Loss, and Vision Impairment --- United States, 2003

RLYB212: Novel Product Candidate to Prevent Maternal Alloimmunization and FNAIT

Descriptive name	Fully human monoclonal anti-HPA-1a IgG
Mechanism of Action	Effect rapid elimination of fetal HPA-1a antigen from the maternal circulation, thereby preventing alloimmunization
Route of Administration	Small volume, SC injection administered no more than once a week; patient friendly auto-injector suitable for self-administration
Pharmacokinetic Results	Limited peak-to-trough variation
Manufacturing	Standard manufacturing for monoclonal antibody
Supply	Robust and stable supply opportunities
Regulatory Designations	ODD (FDA, EMA), Rare Pediatric Disease Designation (FDA)
Key Intellectual Property	Composition of Matter patents

Dose-Dependent Elimination of HPA-1a Platelets by Subcutaneous RLYB212, a Monoclonal Antibody to Prevent Fetal and Neonatal Alloimmune Thrombocytopenia

Christof Geisen[1], Erika Fleck[1], Stephan Martin Gastón Schäfer[2], Carmen Walter[2], Susanne Braeuninger[1], Jens Søndergaard Jensen[3], Róisín Armstrong[4], Douglas Sheridan[4], Kiran Patki[4], Mette Kjaer[5,6], Frank Behrens[2], Erhard Seifried[1], Jens Kjeldsen-Kragh[7,8], Michaela Köhm[2]

[1]Institute of Transfusion Medicine and Immunohaematology, German Red Cross Blood Transfusion Service Baden-Württemberg-Hessen GmbH, Frankfurt am Main, Germany; [2]Fraunhofer Institute for Translation Medicine and Pharmacology ITMP, Frankfurt am Main, Germany; [3]Larix A/S, Herlev, Denmark; [4]Rallybio, New Haven, Connecticut, United States; [5]UiT – the Arctic University of Norway, Hammerfest, Norway; [6]Finnmark Hospital Trust, Hammerfest, Norway; [7]Department of Clinical Immunology and Transfusion Medicine, University and Regional Laboratories, Lund, Sweden; [8]Department of Laboratory Medicine, University Hospital of North Norway, Tromsø, Norway

Presented at the 31st Congress of the International Society on Thrombosis and Haemostasis; June 24-28, 2023; Montréal, Canada.

Disclosures for CHRISTOF GEISEN

- Grants/research support: **None**
- Speakers' bureau or advisory board memberships: **None**
- Patents for drugs or devices: **Antibody Detection Method and System Patent**
- Other: **Employee of German Red Cross**

Presentation Learning Objectives

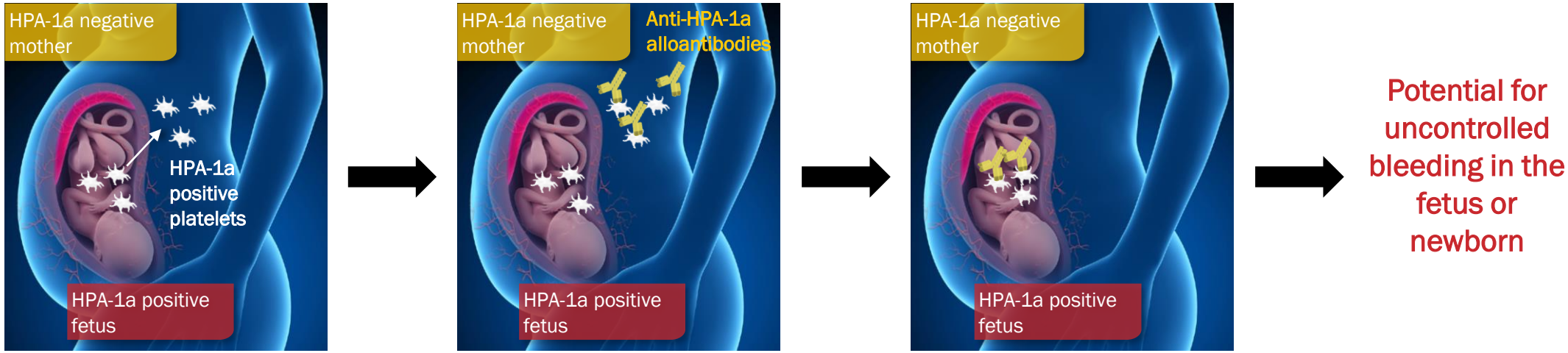
At the conclusion of this presentation, participants will be able to:

- **Describe** FNAIT and recognize that FNAIT is analogous to HDFN, with the distinction that it may occur during the first pregnancy
- **Summarize** data demonstrating that RLYB212 exhibits rapid and dose-dependent elimination of circulating antigen positive cells and **explain** why this is expected to prevent maternal alloimmunization
- **Evaluate** the potential use of RLYB212 as an effective prophylactic approach for pregnancies at risk of FNAIT

FNAIT, fetal and neonatal alloimmune thrombocytopenia; HDFN, hemolytic disease of the fetus and newborn; HPA, human platelet antigen; MOA, mechanism of action.

FNAIT Is the Platelet Counterpart to HDFN[1]

Both are caused by antigen mismatch between maternal and fetal cells[1]



HPA-1a positive platelets from the fetus enter maternal circulation

Immune system of the **mother triggered to produce anti-HPA-1a alloantibodies**

Maternal alloantibodies **cross the placenta** and **destroy HPA-1a positive fetal platelets**

Important Distinguishing Features

Fetal thrombocytopenia may occur in the **first pregnancy**[2]

Alloantibodies may be detected early in the **second trimester**[3]

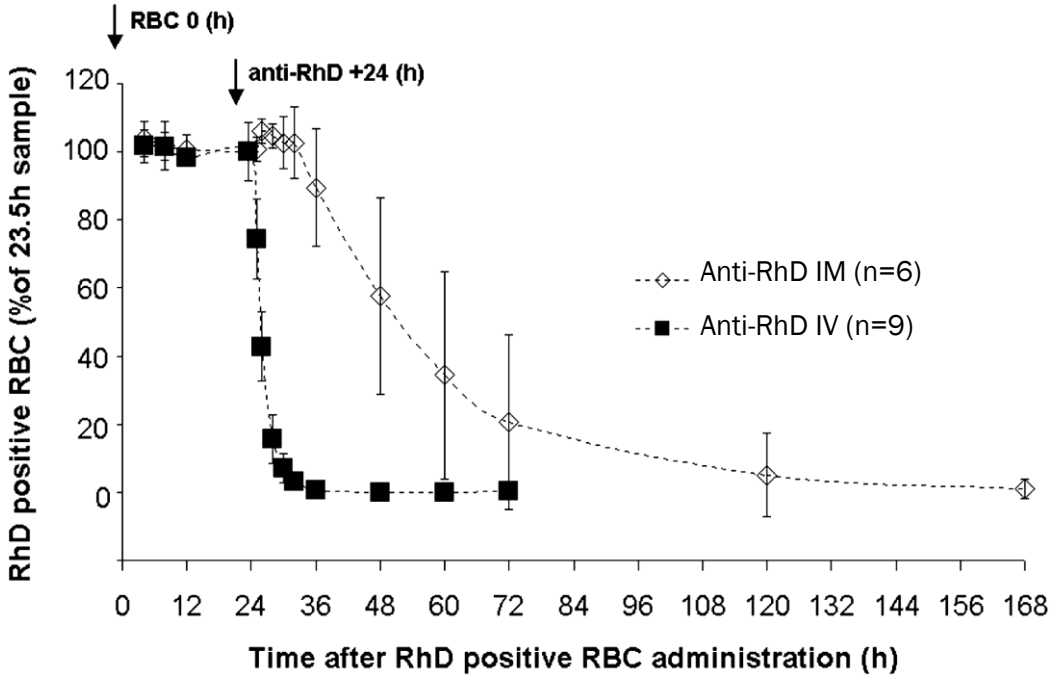
No treatments exist to prevent HPA-1a alloimmunization

FNAIT, fetal and neonatal alloimmune thrombocytopenia; HDFN, hemolytic disease of the fetus and newborn; HPA, human platelet antigen.

1. Bussel JB, et al. *Am J Obstet Gynecol*. 2021;225(2):120-127. 2. Jin JC, et al. *Am J Hematol*. 2019;94(8):213-215. 3. Williamson LM, et al. *Blood.* 1998;92(7):2280-2287.

Precedent for Preventing Maternal Alloimmunization Is Well Established by IM and IV Anti-RhD Treatment

Kinetics of concentration of RhD-positive RBCs (mean ± SD) following IV or IM administration of 1500 IU (300 µg) anti-RhD in healthy RhD-negative males



Reprinted from *Blood*, 103, Miescher S, et al, A single recombinant anti-RhD IgG prevents RhD immunization: association of RhD-positive red blood cell clearance rate with polymorphisms in the FcγRIIA and FcγIIIA genes, 4028-4035, Copyright (2004), with permission from Elsevier

- More than 95% of RhD positive RBCs cleared within 8 hours of IV administration of anti-RhD[1]
- After IM administration of anti-RhD, ~10% of the RhD-positive RBCs cleared in 12 hours and 95% cleared in 4 days[1]
- Anti-RhD prophylaxis is highly effective when administered as an IM injection (up to 72 hours postpartum)[2,3]

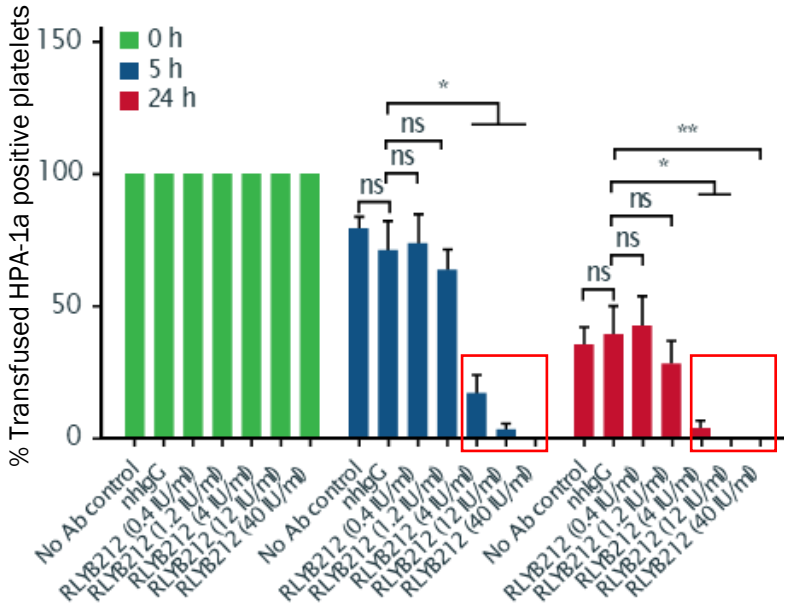
IM, intramuscular; IV, intravenous; RBC, red blood cell.

1. Miescher S, et al. *Blood*. 2004;103(11):4028-4035. 2. Crowther C, et al. Cochrane Database Syst Rev. 1997;(2):Cd000021. 3. RhoGAM. Prescribing information. Kedrion Biopharma. Inc.; March 2019.

In a Preclinical FNAIT Model, RLYB212 Eliminated Platelets at Doses That Prevent Alloimmunization

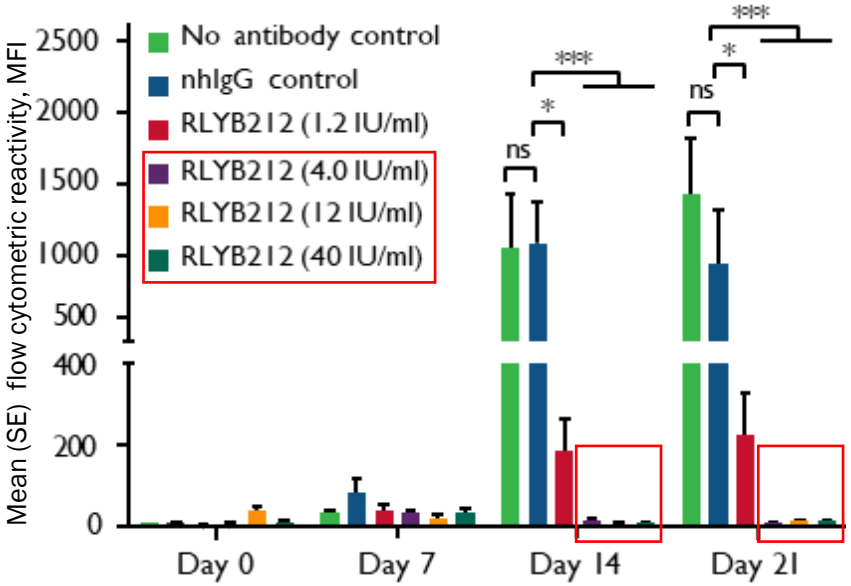
RLYB212 is a novel, recombinant human anti-HPA-1a monoclonal antibody developed to prevent HPA-1a alloimmunization and FNAIT[1]

Elimination of transfused HPA-1a positive platelets



- In a **murine model** of FNAIT, **concentrations of RLYB212 ≥4 IU/mL drove complete elimination** of transfused HPA-1a positive platelets **within 24 h** and **prevented alloimmunization**[1]
- ~4 IU/mL of RLYB212 binds to <10% of the HPA-1a antigen,[1] consistent with the clinical prophylactic treatment paradigm for anti-RhD[2]

Prevention of alloimmunization



Murine model of FNAIT + RLYB212 → Platelet clearance ✓ Prevention of alloimmunization ✓

Figures originally published in [1]. © the American Society of Hematology.
FNAIT, fetal and neonatal alloimmune thrombocytopenia; HPA, human platelet antigen; IgG, immunoglobulin G; nhIgG; normal human immunoglobulin G.
1. Zhi H, et al. *Blood.* 2022;140(20):2146-2153. 2. Brinc D, Lazarus AH. *Hematology Am Soc Hematol Educ Program.*2009;2009(1):185–191.

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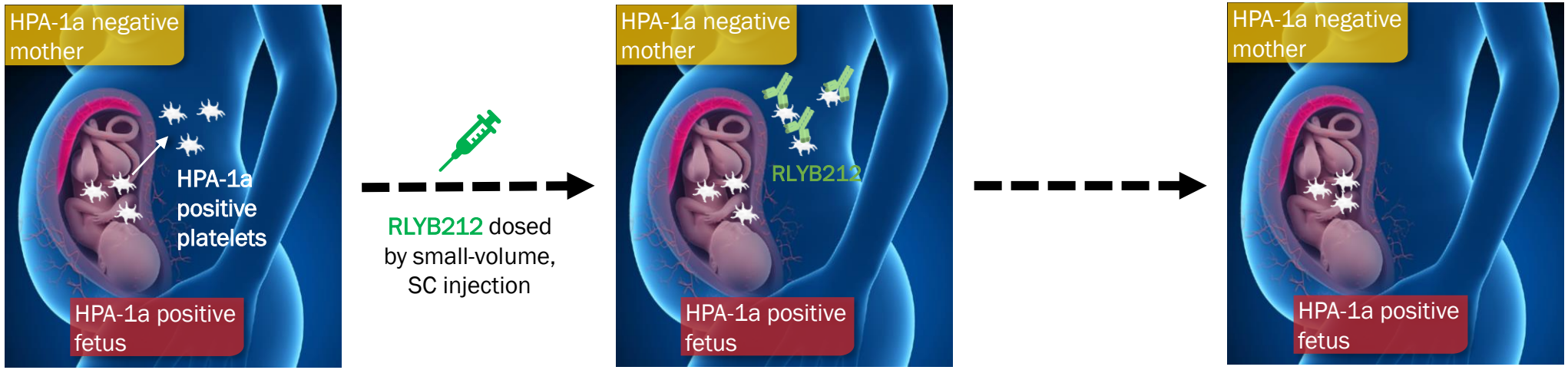
RLYB212, a Novel Candidate for Prevention of HPA-1a Alloimmunization and FNAIT



Prophylactic low-dose SC **RLYB212** is **intended** to drive **rapid elimination** of **fetal**-derived HPA-1a positive **platelets** in maternal circulation

Potentially prevent maternal anti-HPA-1a alloantibody formation and the catastrophic consequences of FNAIT

Here we report results from a **clinical proof-of-concept study** investigating the **capacity of SC RLYB212** to **eliminate HPA-1a positive platelets** transfused to **HPA-1a negative subjects**

FNAIT, fetal and neonatal alloimmune thrombocytopenia; HPA, human platelet antigen; SC, subcutaneous.

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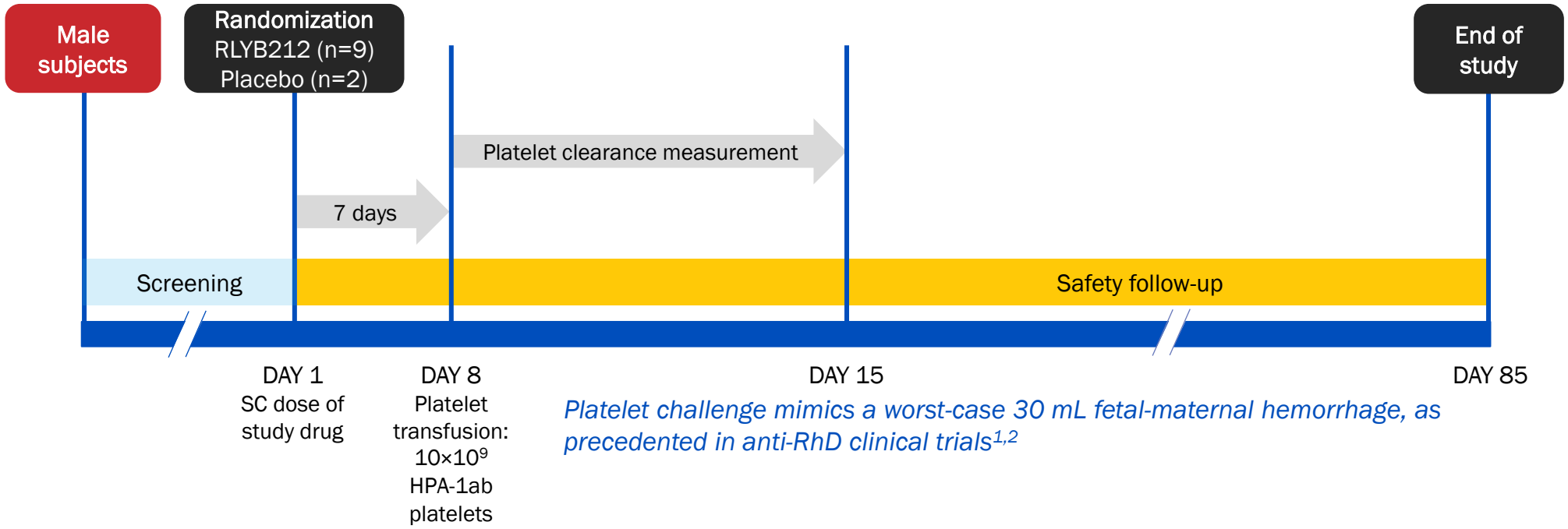
Proof-of-Concept Study Design and Objectives

Phase 1b, randomized, single-blind, placebo-controlled study



Platelet challenge mimics a worst-case 30 mL fetal-maternal hemorrhage, as precedented in anti-RhD clinical trials[1,2]

Eligibility criteria

- Males aged 18 to 65 years
- HPA-1a negative and HLA-A2 negative
- BMI <35 kg/m^2

Primary Objective

- Assess the ability of SC RLYB212 to rapidly eliminate transfused HPA-1a positive platelets (PoC defined as ≥90% mean reduction in platelet elimination half-life vs placebo)

Secondary Objectives

- Characterize the RLYB212 concentration-effect relationship
- Evaluate the safety of SC RLYB212

BMI, body mass index; HLA, human leukocyte antigen; HPA, human platelet; SC, subcutaneous.

1. Sebring ES, Polesky HF. *Transfusion*. 1990;30:344-357. 2. Visser GHA, et al. *Int J Gynaecol Obstet*. 2021;152(2):144-147.

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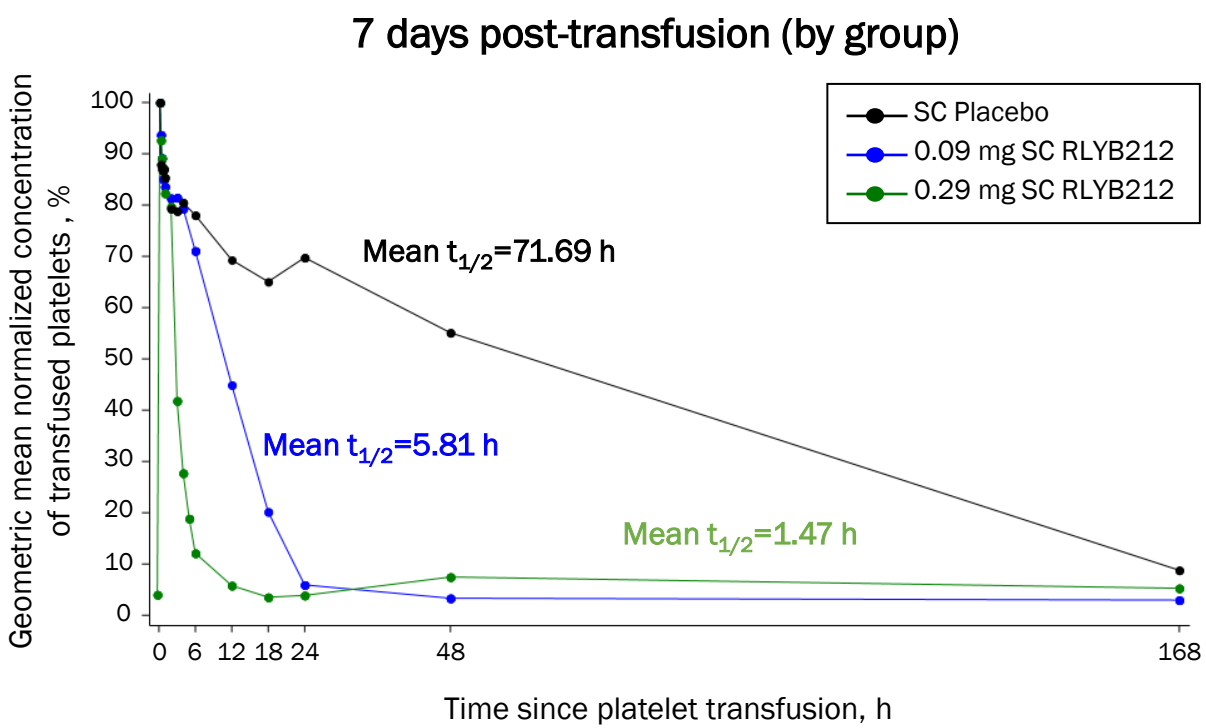
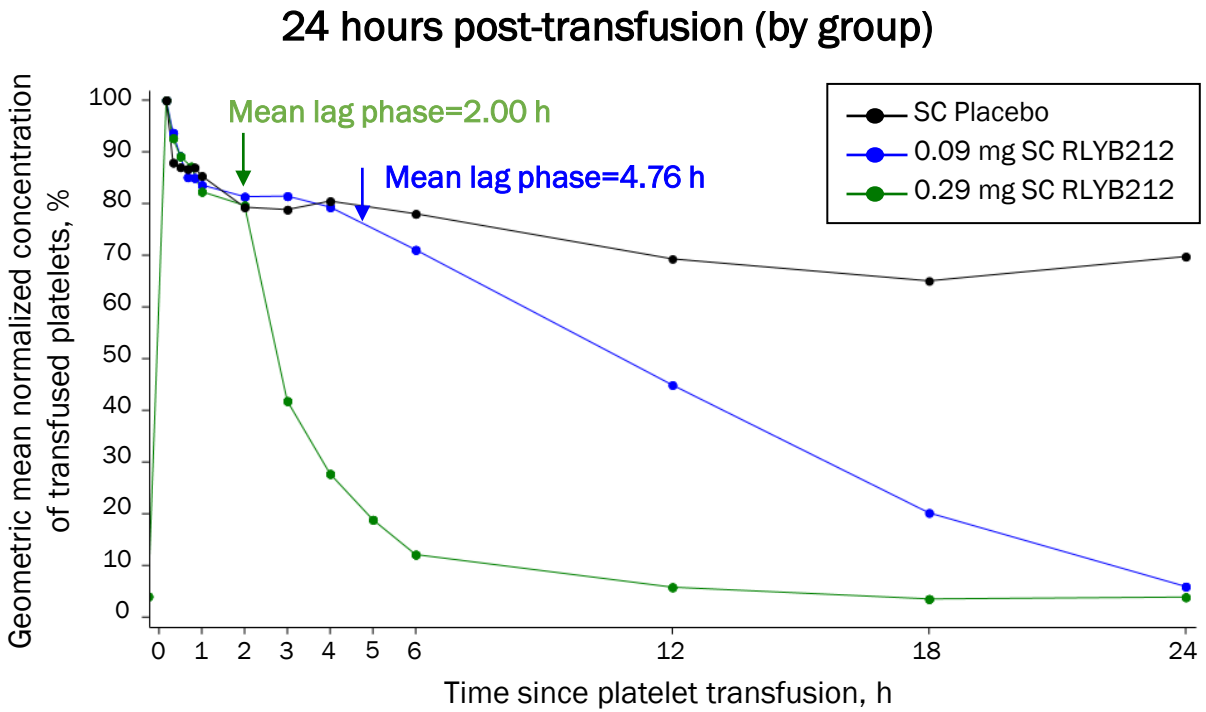
Platelet Elimination Kinetics Were Dose Dependent and Biphasic

Time course of transfused platelet elimination



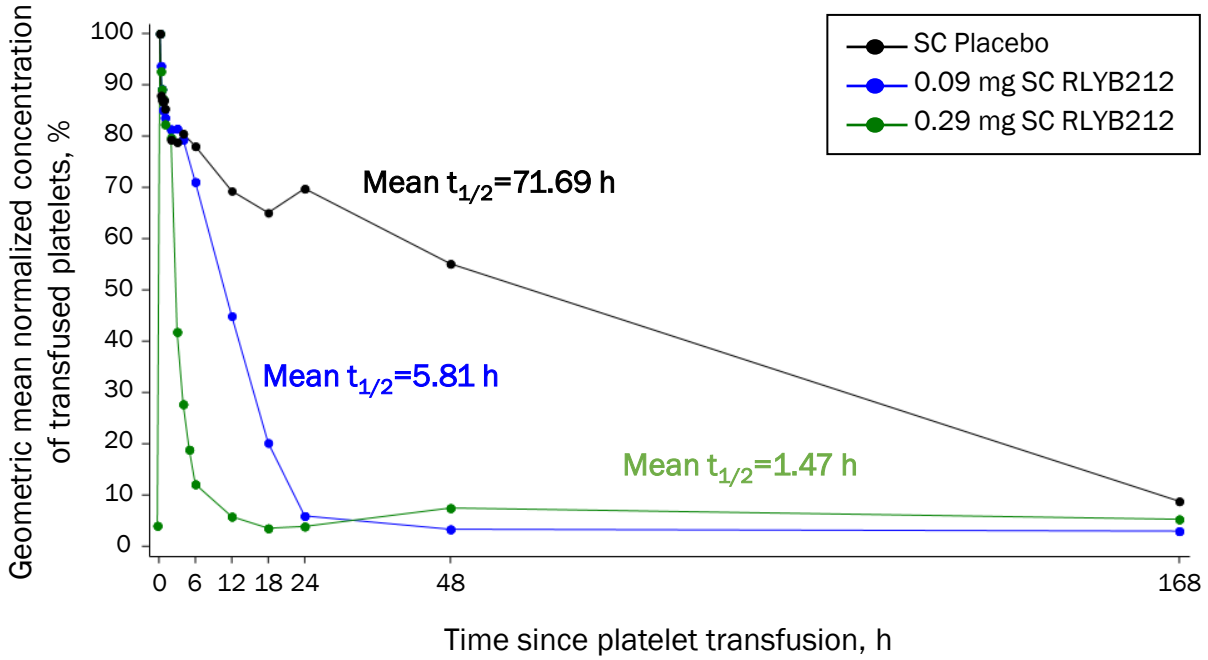


Platelet concentration was normalized at 100% for sample collected 10 minutes after platelet transfusion.
SC, subcutaneous; $t_{1/2}$, half-life.

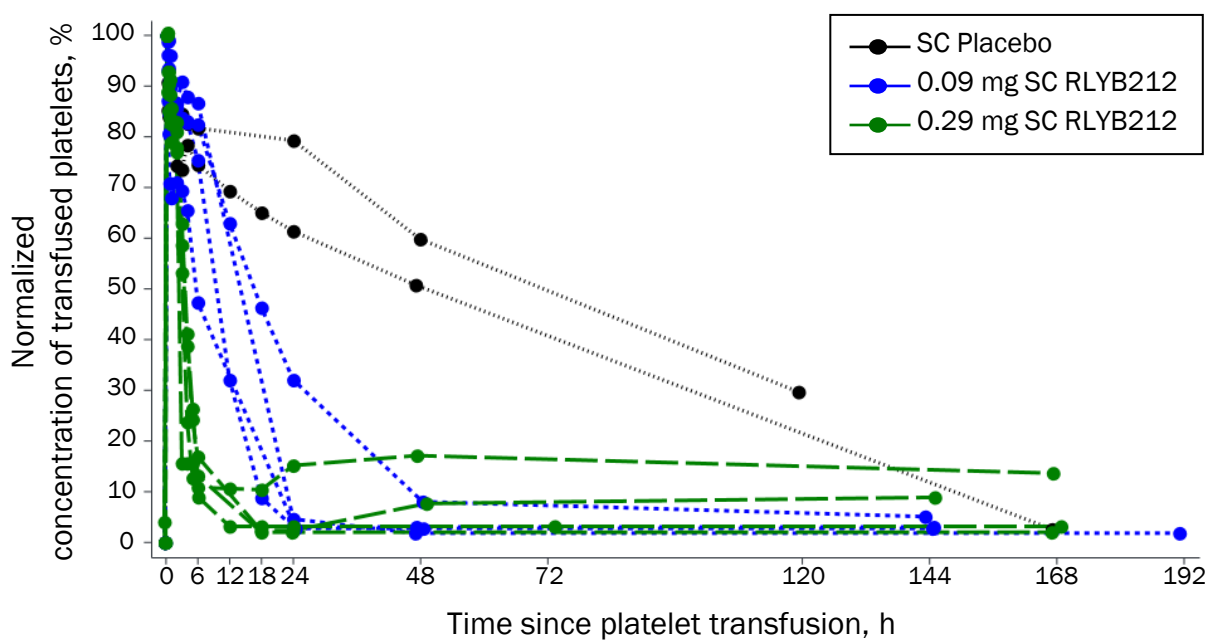
Platelet Elimination Kinetics Were Dose Dependent and Biphasic

Time course of transfused platelet elimination

7 days post-transfusion (by group)



7 days post-transfusion (by subject)



Platelet concentration was normalized at 100% for sample collected 10 minutes after platelet transfusion.
SC, subcutaneous; $t_{1/2}$, half-life.

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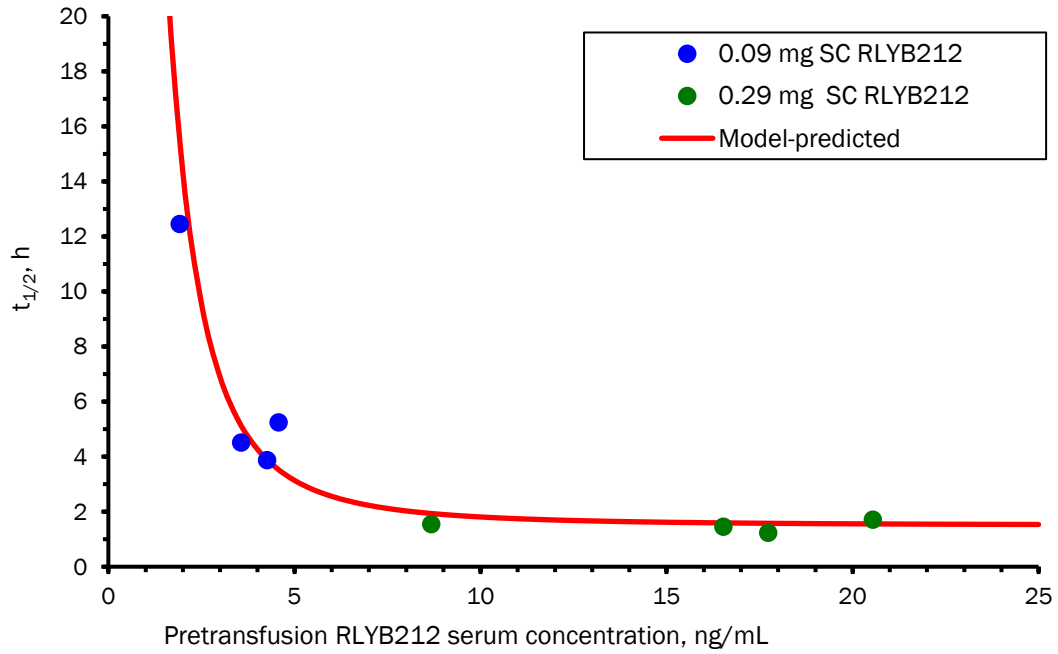
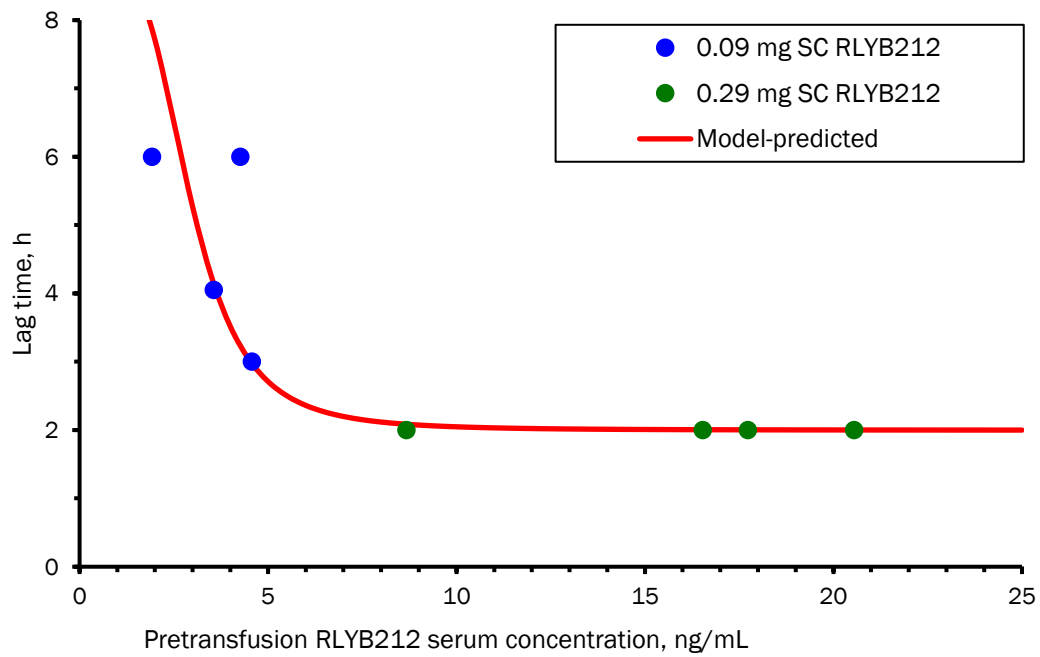
Parameters of Platelet Elimination Kinetics Were SC RLYB212 Concentration Dependent

Platelet elimination half-life vs
RLYB212 concentration before transfusion



Lag time vs
RLYB212 concentration before transfusion



Timing the platelet challenge during the RLYB212 absorption phase and including 2 SC dose levels expanded the range of RLYB212 concentrations before transfusion, resulting in a clear picture of the concentration-effect relationship.

SC, subcutaneous; $t_{1/2}$, half-life.

SC RLYB212 Was Well Tolerated

No reports of:

- Related or possibly related AEs
- Severe or serious AEs

AE, adverse event; SC, subcutaneous.

Conclusions

- SC RLYB212 treatment **rapidly and completely eliminated HPA-1a positive platelets** in HPA-1a negative subjects in a concentration-dependent manner
- Both doses of SC **RLYB212 met the prespecified proof-of-concept criteria** of ≥90% mean reduction in platelet elimination half-life

- Platelet elimination kinetics after a single SC dose of RLYB212 were consistent with elimination kinetics of RhD positive erythrocytes after IM administration of anti-RhD agents[1]
- SC RLYB212 was well tolerated
- Collectively, our data support the potential use of SC RLYB212 as a prophylactic for FNAIT

FNAIT, fetal and neonatal alloimmune thrombocytopenia; HPA, human platelet antigen; IM, intramuscular; SC, subcutaneous.

1. Miescher S, et al. *Blood*. 2004;103(11):4028-4035.

ISth 2023

Acknowledgments

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- This study was funded by Rallybio IPA, LLC

PoC Study Q&A

RLYB212

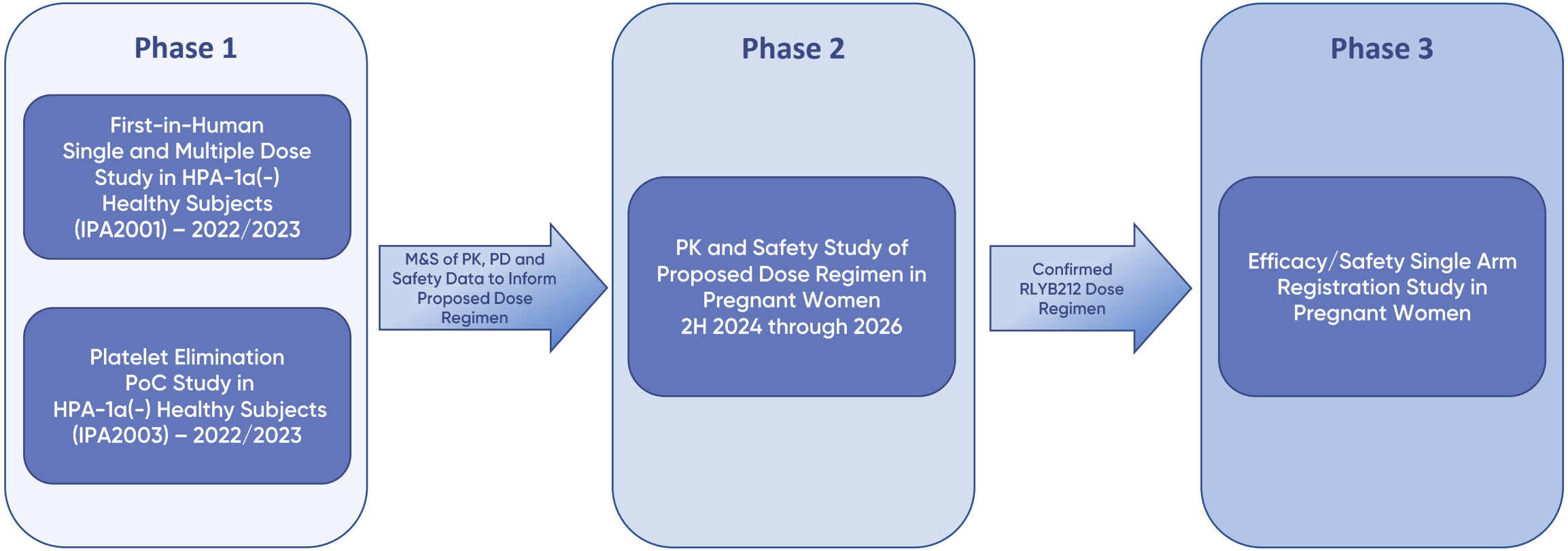
Clinical Development Program

Key 2023 Milestones for RLYB212 Development Plan

✓ Deliver Phase 1b Proof-of-Concept Study Results

- Complete toxicology program, inclusive of maternal-fetal toxicology
  - ON TRACK for 4Q 2023
- Complete multiple dose cohort of Phase 1 safety and PK study
  - ON TRACK for 4Q 2023
- Initiate regulatory discussions to support Phase 2 study
  - ON TRACK for 4Q 2023

RLYB212 Clinical Development Plan



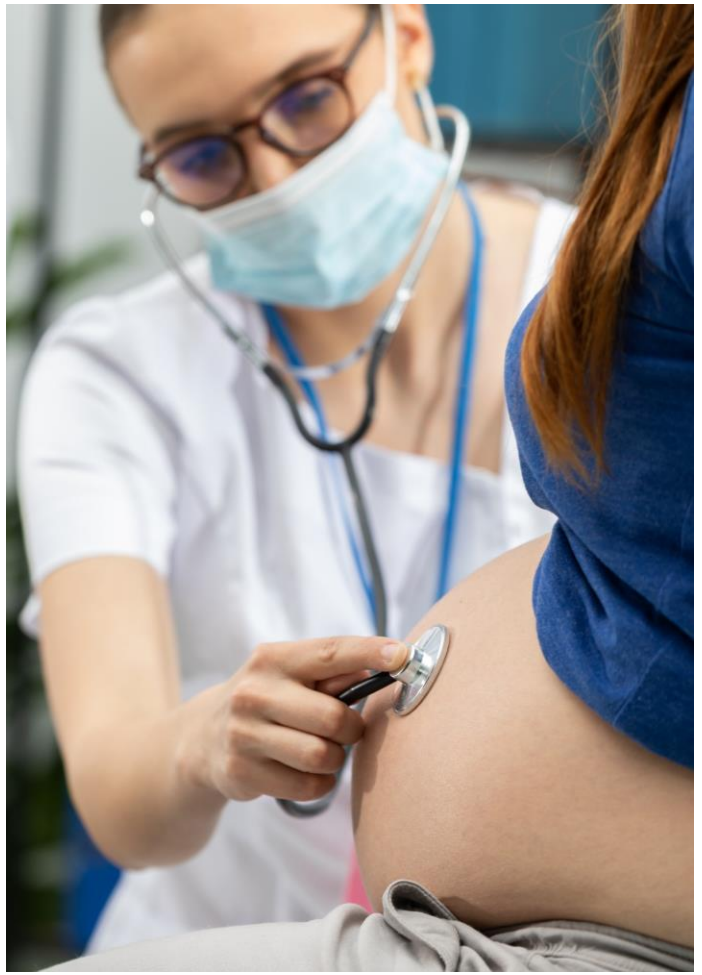
Prospective FNAIT Natural History Study in Pregnant Women
Contemporary Control Dataset for HPA-1a Alloimmunization to Support Single-Arm Registration Study (IPA2002) – through 2026

M&S = modeling and simulation, PoC = Proof of Concept, Pregnant Women = women with HPA1-a mismatched fetus and at higher risk for HPA-1a alloimmunization and FNAIT

RLYB212 Phase 2 Study in Pregnant Women at Higher Risk for Maternal HPA-1a Alloimmunization

Design Principles

- Comprehensive PK to characterize systemic exposure through pregnancy and at parturition
- Sentinel dosing with sequenced cohorts
- Safety assessed for mother, fetus and newborn, including pregnancy and neonatal outcomes
- Monitor post partum for occurrence of maternal HPA-1a alloimmunization



Phase 2 Dose Confirmation Study in Pregnant Women

Expected to Start in 2H 2024

STUDY DESIGN

Single-arm, open-label study to assess the pharmacokinetics (PK) and safety of subcutaneously (SC) administered RLYB212 in 8 pregnant women at higher risk for HPA-1a alloimmunization

To be conducted at European sites

STUDY POPULATION/TREATMENT

HPA-1 a negative and HLA-DRB3*0101 positive pregnant women bearing an HPA-1a positive fetus, with no existing alloimmunization

Dosing of RLYB212 initiated by Gestation Week 16 and continued at regular intervals through pregnancy

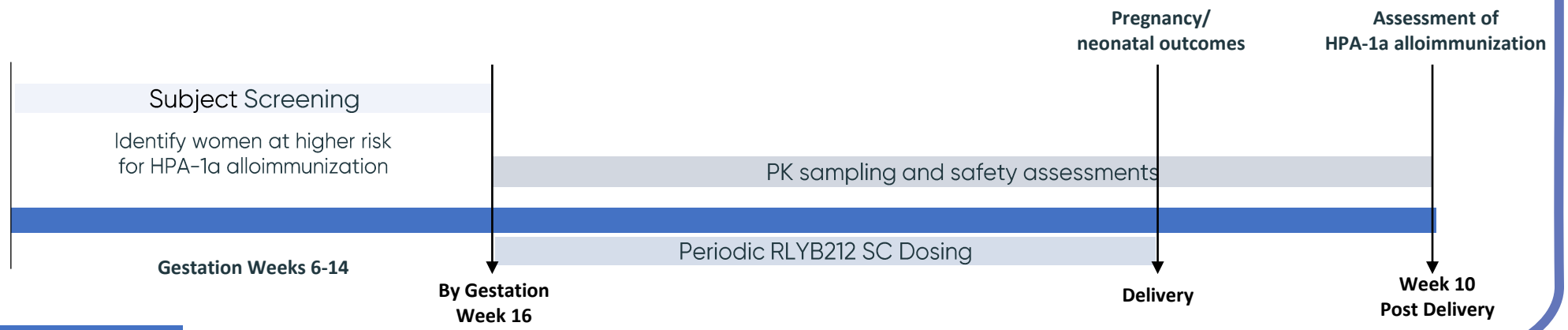
PRIMARY OBJECTIVE

To assess the PK and safety of SC RLYB212 administered antenatally in pregnant women

SECONDARY OBJECTIVES

To assess the safety of RLYB212 in the neonate and neonatal exposure of RLYB212 at time of birth

To assess pregnancy and neonatal outcomes and the occurrence of emergent HPA-1a alloimmunization



Study Design

FNAIT Natural History Study

To Be Continued in Parallel with Phase 2 Study

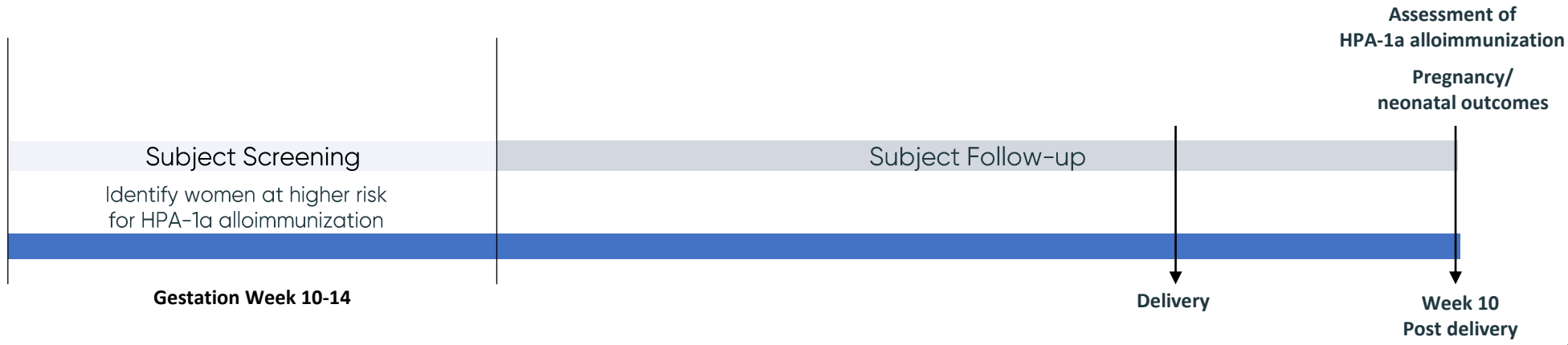
STUDY DESIGN

Prospective, non-interventional, multinational natural history study across ~ 30 sites in US/EU [1]

Screen expectant mothers presenting for Gestation Week 10 to 14 prenatal visit for higher FNAIT risk (HPA-1a negative and HLA-DRB3*01:01 positive)

OBJECTIVES

- Provide historical alloimmunization rate to serve as control dataset for planned, single-arm, Phase 3 registrational study
- Obtain prevalence estimates of the FNAIT at-risk population, including racial and ethnic groups under-represented in previously published studies
- Establish operational scaffold for RLYB212 interventional studies, including implementation of lab tests to screen for higher FNAIT risk

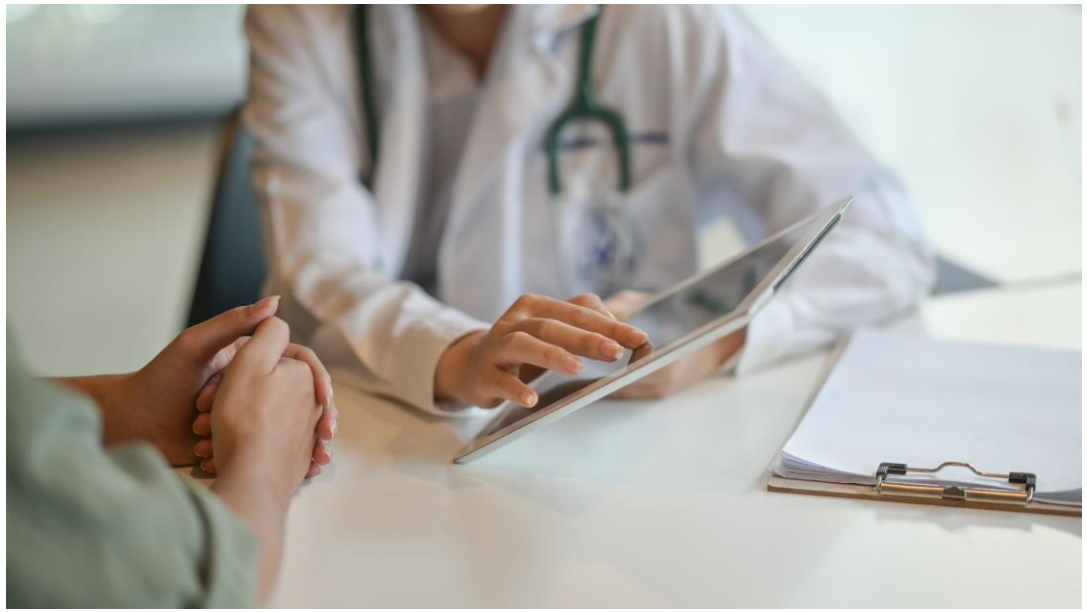


Study Design

1. ~20 - 30K to be screened

Natural History Study: Current Experiences

- Women are willing to be screened in early pregnancy for risk of HPA-1a alloimmunization, and FNAIT
- Approximately 4,500 women screened to date
- Race and ethnicity of the screened population is are generally representative of US and EU populations
- Frequency of HPA-1a negative gene status is generally consistent with published literature (~2%)
- Women with existing HPA-1a alloimmunization are also being identified
  - Prophylactic anti-HPA-1a, if available, may have prevented these cases



Natural History Study: Areas of Focus

- Maintain screening in parallel with Phase 2 execution, fulfilling the study's role as an operational precedent to seamless initiation of the registration trial
  - On track for ~7,500 women to be screened by year end 2023
- Work with current EU sites to prepare for introduction of the Phase 2 study
  - Target population of the Natural History Study same as that for the Phase 2 study/future registration study
- Expand US site footprint to support overall screening target for 2026, with US race / ethnicity representative of the broader US population

RLYB212 Regulatory Interactions and Overview

- RLYB212 development based on phase-appropriate regulatory guidance and includes specific feedback on:
  - Maternal-fetal toxicology program
  - Core design elements of the clinical program
- EMA Scientific Advice/Protocol Assistance planned for Phase 2 study
  - Preceding local regulatory applications for participating Phase 2 countries
- EOP2 discussions for Phase 3 registration study will occur after Phase 2, when:
  - Dose regimen confirmed for RLYB212
  - Control dataset established for HPA-1a alloimmunization

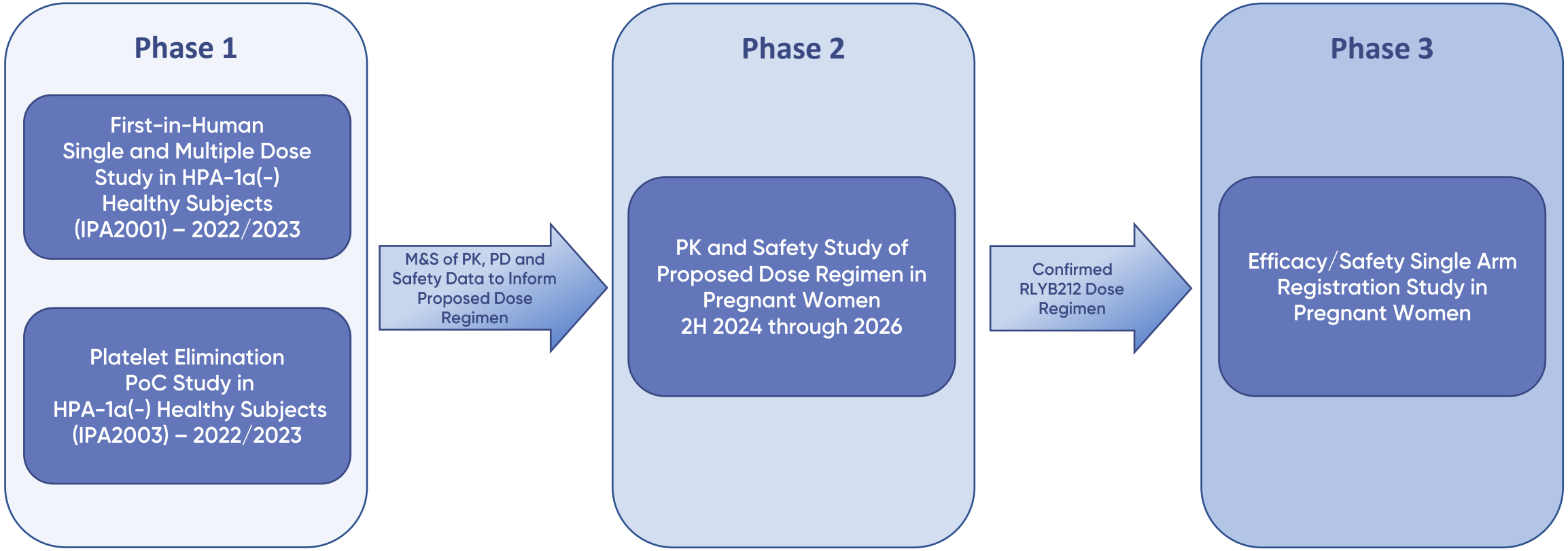
Orphan Drug designation by EMA (April 2020) and FDA (July 2020)

Rare Pediatric Disease designation by FDA (March 2020)

Key Elements for a Successful Registration Program

- Phase 1/2:
  - Proof-of-concept established for accelerated platelet elimination
  - Robust safety margins established from maternal fetal toxicology program
  - Therapeutic dose regimen confirmed
- Natural History Study:
  - Establish contemporary control dataset for HPA-1a alloimmunization
- Regulatory:
  - Complete end-of-Phase 2 regulatory discussions (US and EU)
- CMC:
  - RLYB212 formulated in patient-friendly SC delivery system
- Partner with world-class IVD partner for FNAIT screening tests

RLYB212 Clinical Development Plan



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Upcoming 2023 Catalysts

RLYB212

- Phase 1 Multiple Dose Safety and PK Data 4Q2023
- Maternal Fetal Toxicology Program Completes 4Q2023

RLYB116

- Phase 1 Multiple Ascending Dose Study of RLYB116 Safety, PK and PD Data Expected in 4Q 2023
- RLYB116 Indication Strategy Expected in 4Q 2023

Management Q&A


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