A SCALABLE GENE THERAPY PLATFORM TARGETING RARE MONOGENIC DISEASES

APRILIGEN

AS IF EVERY CHILD IS OUR OWN

APRILIGEN, INC.

YALE INNOVATION SUMMIT PITCHFEST

MAY 2025

www.apriligen.com

APRILIGEN, INC.

Apriligen is commercializing its proprietary ex vivo HSC (Hematopoietic Stem Cell) lentiviral gene therapy backbone across a range of ultra-rare indications beginning with Diamond Blackfan Anemia (“DBA”)

Team	• Proven organizational leadership, science and governance
Science	• Clinically adjacent, patent protected lead asset validates platform technology
Regulatory	• Novel trial design, potential for accelerated approval in ‘26 • First DBA patient dosed in 2H 2025
Market	• Thousands of patients in US and EU DBA registries; curative potential with no near-term competitive therapy

ORGANIZATIONAL LEADERSHIP: EXPERIENCE AND EXPERTISE

- Leaders Dedicated to Apriligen's Mission with Proven Business, Scientific and Gene Therapy Development Expertise
- Founder families are impacted by rare disease and have invested over $17.5M of personal capital into developing Apriligen – aligning interests with investors

Founders

Goldman Sachs

Boston Children's Hospital

NIH National Institutes of Health

Columbia Care

MHH Medizinische Hochsch Hannover

Lund University

Operational Excellence

Gene Therapy Program – Perelman School of Medicine, University of Pennsylvania

Solutions RNRBIOMEDICAL

Independent Governance

CBIT Yale Center for Biomedical Innovation and Technology

SickKids The Hospital for Sick Children

Alexion

Goldman Sachs

Achillion

Scientific Advisory Board

Dana-Farber Cancer Institute | Boston Children's

Dana-Farber/Boston Children's Cancer and Blood Disorders Center

Stanford Medicine

NHS Imperial College Healthcare NHS Trust

Vector & Plasmid Manufacturing

Lentigen Technology Inc.

a Miltenyi Biotec Company

aldevron™

Process Development / CMC Optimization

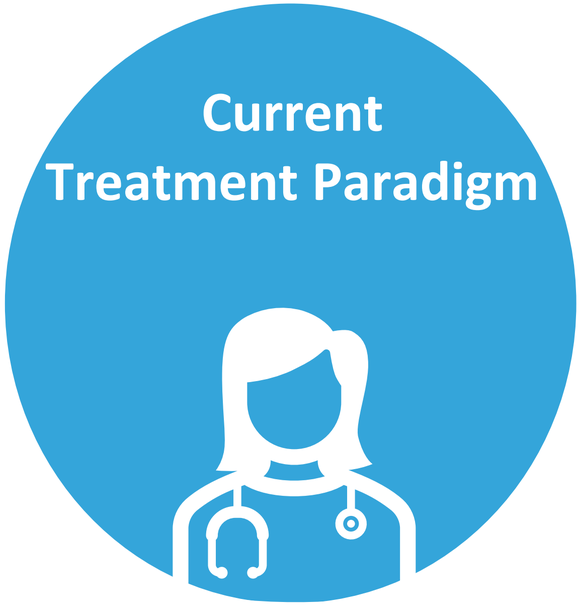
charles river

Minaris Regenerative Medicine

DBA: STANDARD OF CARE IS PART OF THE PROBLEM

- **Chronic blood transfusions and concurrent iron chelation therapy**
- **Corticosteroids**
- **Hematopoietic stem cell transplantation (HSCT)**

<u>The current standard of care can result in infection, organ failure, GVHD, treatment related fatality</u>



(1) Source: US Diamond Blackfan Anemia Registry.

OPPORTUNITY: UNMET MEDICAL NEED IN DBA

- **Illness presents during first 45 days of life**
- **< 5,000 patients are living with DBA worldwide – increasing every year**
- **Over 18 distinct, ribosomal variants with similar clinical profile**

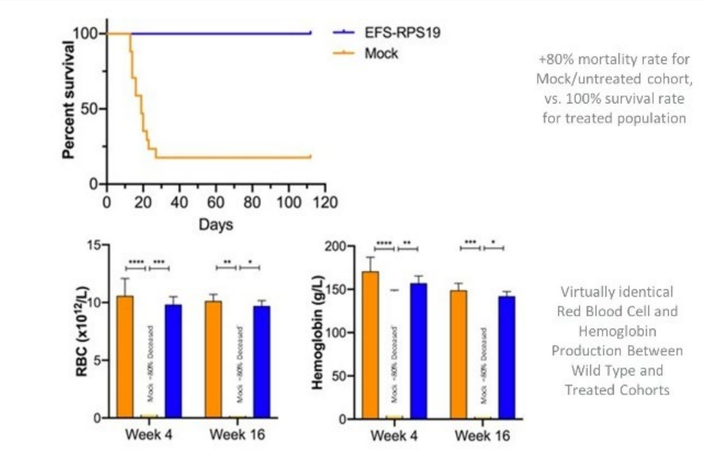
DBA patients die young, require intensive, ongoing medical care and have materially increased risk for solid tumor and blood cancers

1. US Diamond Blackfan Anemia Registry, Feinstein Institute for Medical Research; 2. RPS-19 (~35%), RPL-5 (~12%), RPS-26 (~9%), RPL-11 (~7%), RPL35a (~3%), RPS-17 (~4%), RPS-24 (~3%), RPS-10 (~1%), other (~15%).

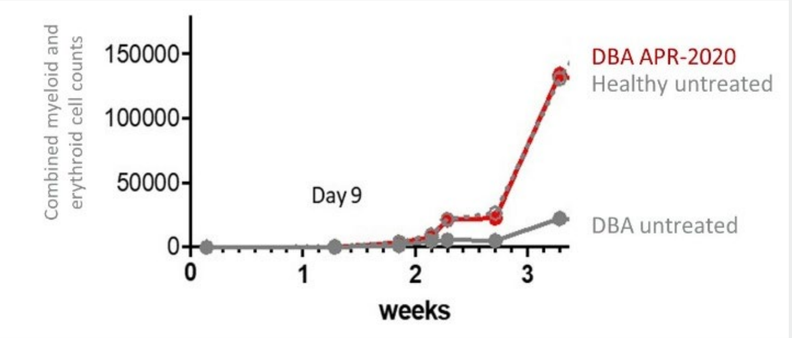
APRILIGEN'S SOLUTION: ROOTED IN DATA – SAFE AND EFFICACIOUS

Our next generation, clinically-validated self-inactivating lentiviral vector overcomes hurdles from earlier LVV constructs

Consistent, long-term in vivo correction of anemia and bone marrow failure



Expectations Exceeded in ex vivo patient cells



REGULATORS WANT PLATFORM SOLUTIONS TO CURE RARE GENETIC DISEASES

Improved/Accelerated Approval Pathways for Ultra-Rare:

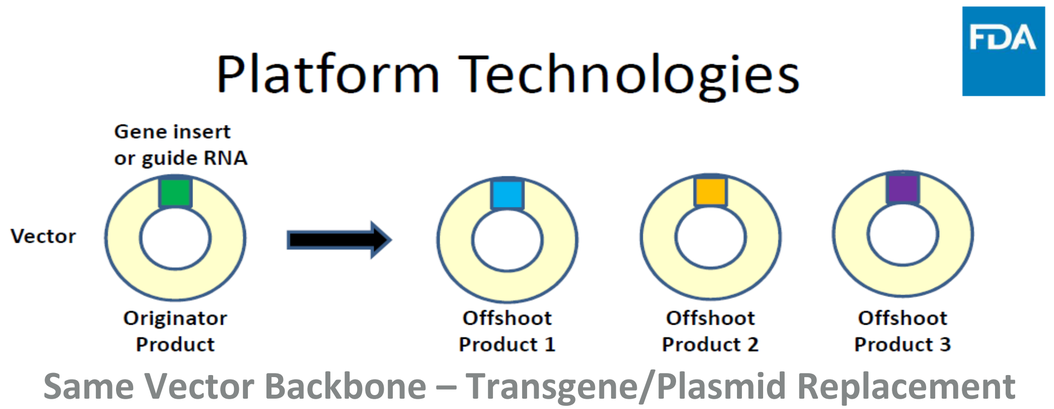
"…we're going to be rolling out a new pathway for drugs, which is a pathway based on a plausible mechanism. If there's a rare condition or a condition that's incurable that affects a small number of people, we may be approving drugs based on a plausible mechanism on sort of a conditional basis."

FDA Commissioner Martin Makary, MD

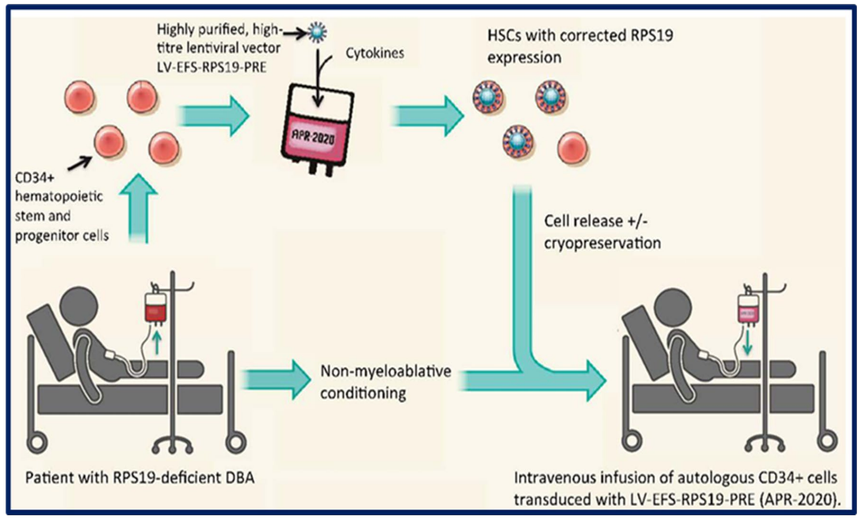
FDA's Case For Platform Technologies:

"In appropriate situations, non-clinical data and manufacturing information from one product may be able to be leveraged to another."

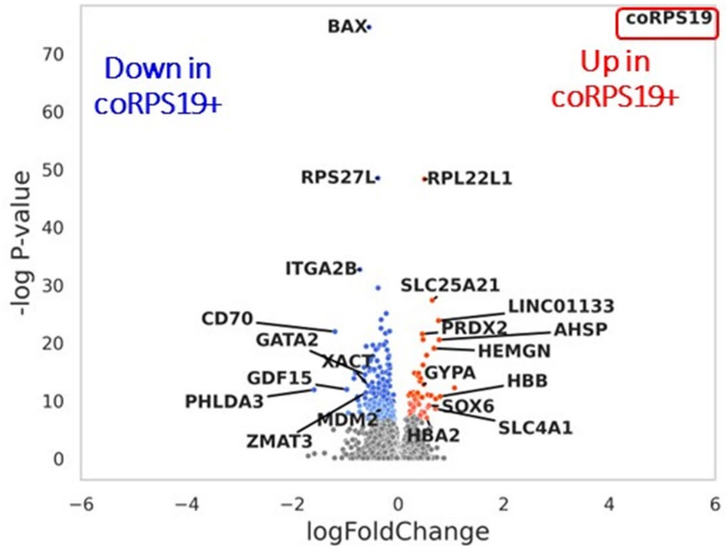
Dr. Peter Marks, Former Director CBER, FDA



OUR SOLUTION – CLINICAL DATA IN 2025



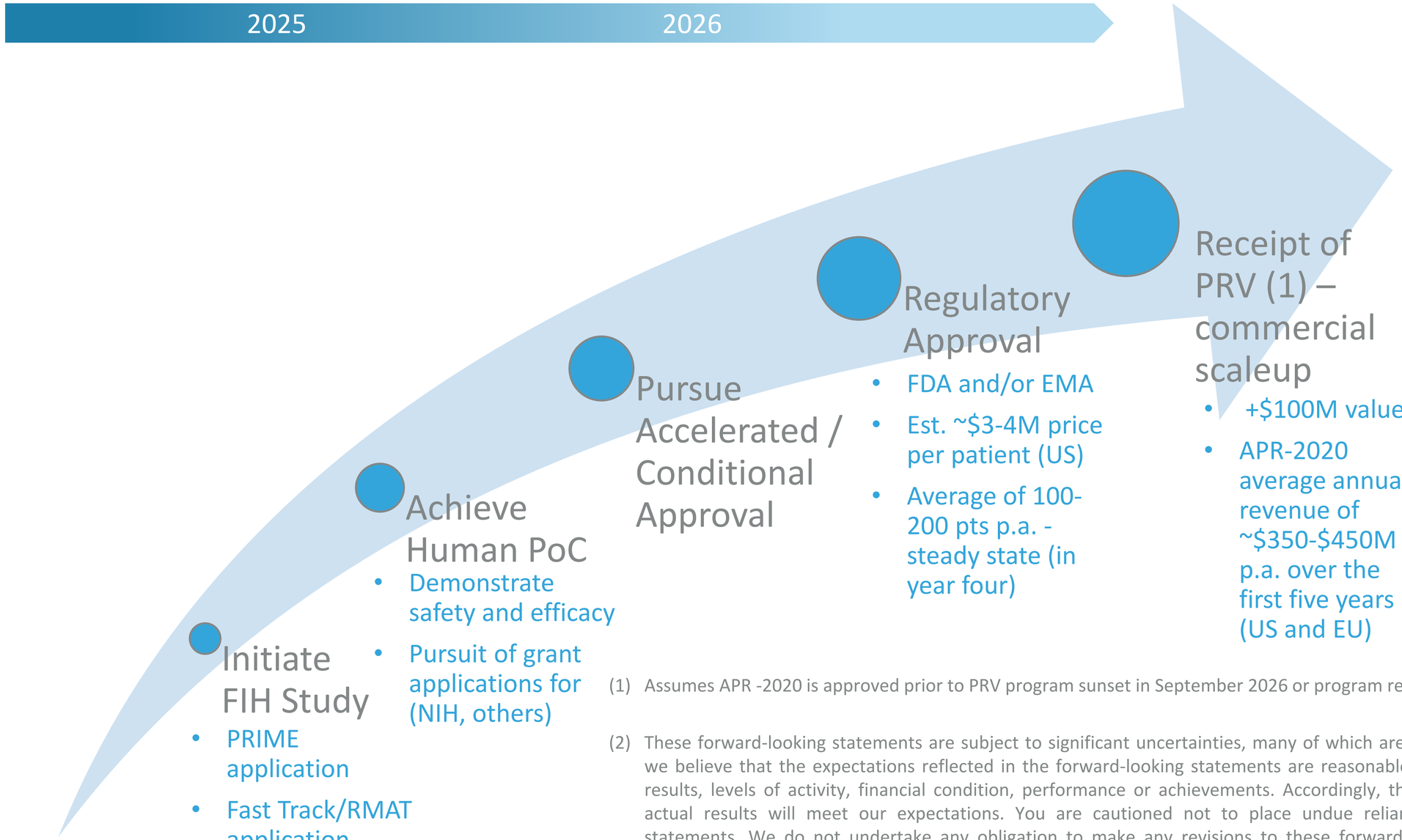
Clinical and Mechanistic Correction: APR-2020 Normalized Gene Activity



Establishing a new, curative front-line therapy

- No ablative chemotherapy, safer conditioning than myeloablative standard
  - Reducing conditioning related side effects
- Autologous - No marrow match/donor necessary (No Graft vs. Host reactions)
- No immunosuppressants & risk of immunocompromised infections
- Substantial reduction in cancer-related chemo risk/functional stresses
- Attractive reimbursement and health economic impact
- One and done correction

KEY VALUE INFLECTION POINTS & MILESTONES



(1) Assumes APR -2020 is approved prior to PRV program sunset in September 2026 or program reauthorization.

(2) These forward-looking statements are subject to significant uncertainties, many of which are beyond our control. Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee future results, levels of activity, financial condition, performance or achievements. Accordingly, there can be no assurance that actual results will meet our expectations. You are cautioned not to place undue reliance on these forward-looking statements. We do not undertake any obligation to make any revisions to these forward-looking statements to reflect circumstances after the date hereof or to reflect the occurrence of unanticipated events.

MONOGENIC RARE DISEASE PLATFORM - SIGNIFICANT UNMET MEDICAL NEED

Apriligen is developing its proprietary HSC (Hematopoietic Stem Cell) lentiviral gene therapy backbone across a range of ultra-rare indications

Rare Disease Statistics

- Effects ~5% – 10% of global population, ~25-30M Americans
- 7,000 Rare Diseases, 80% are monogenic
- Disproportionately impacts pediatric population

Rare Disease Characteristics

- Defined at less than 200,000 worldwide in US, fewer than 65 out of 100,000 – ultra rare affects fewer than 1 in 50,000 people
- Resource intensive to manage; significant burden on healthcare system

Rare Disease Solutions

- Genetic problems are best solved by genetic solutions – CGT is the answer
- Innovative regulatory, scientific and manufacturing solutions now exist to facilitate Apriligen's platform solution

JOIN US ON THIS MISSION – SHINE A LIGHT ON THIS OPPORTUNITY

Series A Preferred Use of Proceeds:

- Initiate and Complete pivotal P-I/II trial
- Data Permitting - Submit for Accelerated/Conditional Approval and/or full approval in US and EU

Milestones achieved with funding:

- Demonstrate clinical safety and efficacy needed for APR-2020 regulatory approval and commercialization (commercial launch and Priority Review Voucher monetization)
- Validate platform, select next development candidates for IND-enabling studies
- Application for Conditional/Accelerated Approval (Data Dependent)

Status:

- $15M Institutional raise under way that includes CT Innovations

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