generation bio™

Creating a New Class of **Durable Redosable Gene Therapy**

FOR MILLIONS OF PATIENTS LIVING WITH RARE AND PREVALENT DISEASES

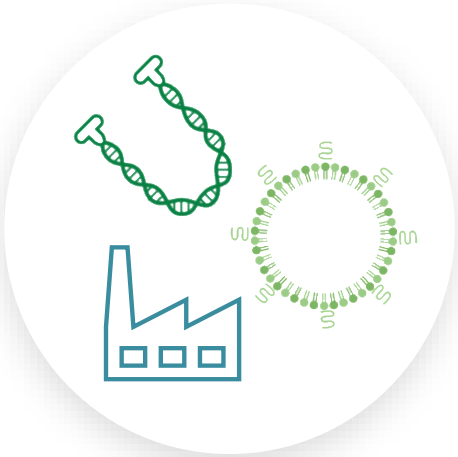
NASDAQ: GBIO

MARCH 2021

Forward-looking statements

Any statements in this presentation about future expectations, plans and prospects for the Company, including statements about the Company's strategic plans or objectives, technology platforms, research and clinical development plans, manufacturing plans and goals, and other statements containing the words "believes," "anticipates," "plans," "expects," and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: uncertainties inherent in the identification and development of product candidates, including the conduct of research activities, the initiation and completion of preclinical studies and clinical trials and clinical development of the Company's product candidates; uncertainties as to the availability and timing of results from preclinical studies and clinical trials; whether results from preclinical studies such as the ones referred to in this presentation will be predictive of the results of later preclinical studies and clinical trials, including whether levels of expression in one species will translate to expected levels of expression in another species; expectations for regulatory approvals to conduct trials or to market products; challenges in the manufacture of genetic medicine products; the Company's ability to obtain sufficient cash resources to fund the Company's foreseeable and unforeseeable operating expenses and capital expenditure requirements; the impact of the COVID-19 pandemic on the Company's business and operations; as well as the other risks and uncertainties set forth in the "Risk Factors" section of the Company's final prospectus for the Company's initial public offering filed pursuant to Rule 424(b)(4) under the Securities Act of 1933, as amended with the Securities and Exchange Commission, the Company's most recent quarterly report on Form 10-Q, which is on file with the Securities and Exchange Commission, and in subsequent filings the Company may make with the Securities and Exchange Commission. In addition, the forward-looking statements included in this presentation represent the Company's views as of the date hereof. The Company anticipates that subsequent events and developments will cause the Company's views to change. However, while the Company may elect to update these forward-looking statements at some point in the future, the Company specifically disclaims any obligation to do so. These forward-looking statements should not be relied upon as representing the Company's views as of any date subsequent to the date on which they were made.

Creating a new class of durable redosable non-viral gene therapy



PROPRIETARY DISRUPTIVE PLATFORM

integrates construct, delivery and manufacturing

RAPID DEVELOPMENT & SCALING ENGINE

addresses rare indications and previously unreachable prevalent diseases

BROAD & EXPANSIVE PORTFOLIO

includes wholly owned programs across multiple tissues

Our proprietary non-viral gene therapy platform

THREE CORE ELEMENTS

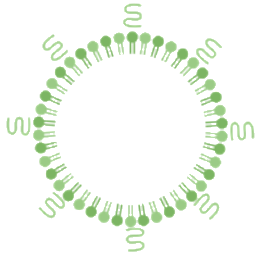
CONSTRUCT | DELIVERY | MANUFACTURING

ceDNA

CLOSED-ENDED DNA

DURABLE EXPRESSION OF LARGE GENETIC PAYLOADS

28 PATENT FAMILIES



ctLNP

CELL-TARGETED LNP

REDOSABLE DELIVERY TO MULTIPLE TISSUES

10 PATENT FAMILIES

MFG

CAPSID-FREE MANUFACTURING

SCALABLE FOR MILLIONS OF PATIENTS

2 PATENT FAMILIES & TRADE SECRETS

Pending patent family numbers current as of 30 November 2020.

As of 30 November 2020 we do not own or exclusively license any issued patents in any jurisdiction.

Generation Bio is poised to unlock the full potential of gene therapy

FEATURE	generation bio
Durable	✓
Redosable	✓
Titratable	✓
Large Genetic Payload	✓
Native Gene Regulation	✓
Tissue Specificity	✓
Large Scale Manufacturing	✓

PATIENTS

- Titration to target expression level for each patient
- Dosing in childhood before disease progression
- Redosing to extend benefit over a lifetime

PHYSICIANS

- Address new indications with larger/multiple genes
- Rescue for undertreated AAV patients
- Extend gene therapy to millions of patients

PAYERS

- More predictable clinical outcome
- Redosing ensures durable benefit
- Reimbursement in current paradigm

Our broad and expansive portfolio

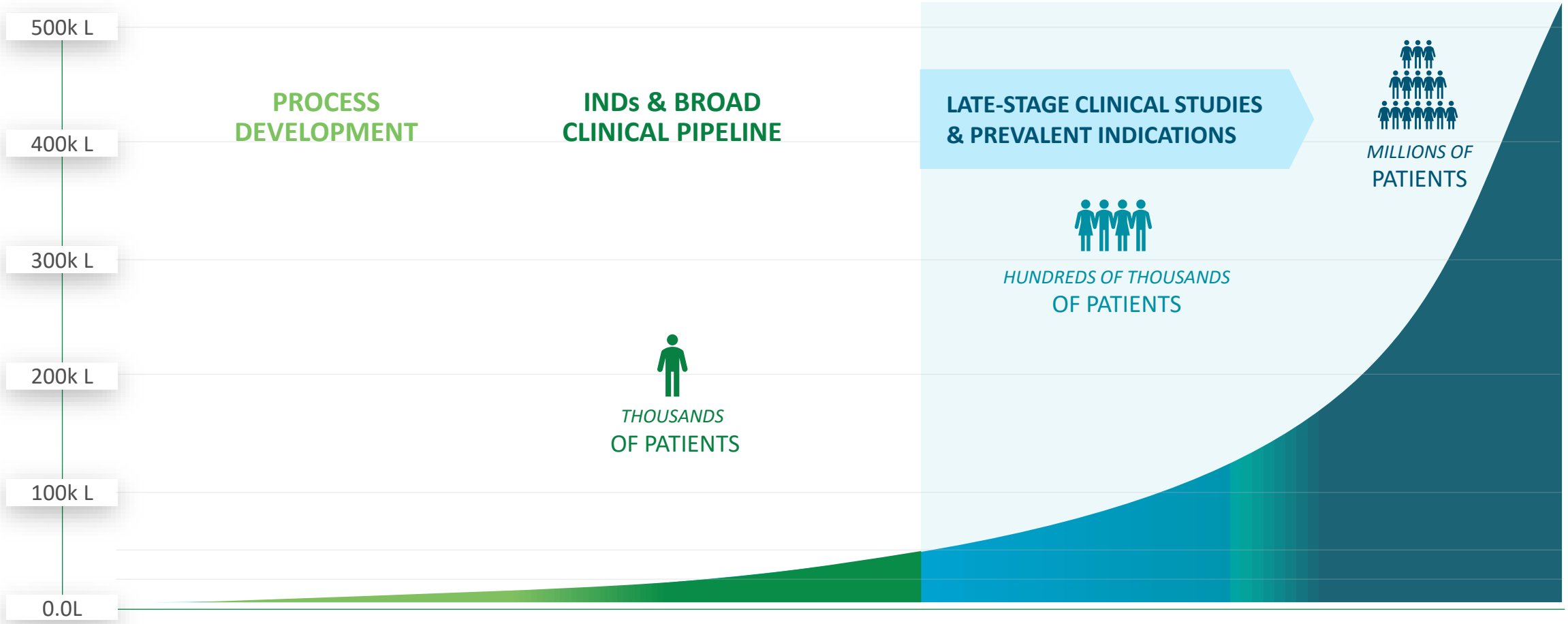
Indications with established endpoints and clear path to human proof of concept

THERAPEUTIC AREA	PROGRAM	US PREVALENCE*	RESEARCH	LEAD OPTIMIZATION	PRE-CLINICAL DEVELOPMENT	PHASE 1 CLINICAL	PHASE 2 CLINICAL	PIVOTAL CLINICAL
Liver	Hemophilia A	16,000						
	PKU	15,000						
	Wilson Disease	11,000						
	Gaucher Disease	6,000						
	Antibody gene therapy							
Retina	LCA10	2,200						
	Stargardt	37,000						
	Wet AMD	1,200,000						

EXPANSION OPPORTUNITIES : Skeletal Muscle | Oncology | CNS

* Prevalence data are approximate

Established scale is sufficient to support broad and expansive clinical pipeline



200L Proprietary capsid-free process

200 to 500L Tech transfer to CMOs with access to multiple 200-500L cGMP suites

1,000L+ SCALE Scaling in progress with access to late-stage GMP site

20,000L+ SCALE Potential facility acquisition

World class team and investors to scale

PROVEN TEAM TRACK RECORD

40+ IND filings, 20 approved products

$470M IN CASH*

to fund innovation

LEADERSHIP TEAM

GEOFFREY **MCDONOUGH, MD**
President & CEO

MATTHEW **NORKUNAS, MD, MBA**
Chief Financial Officer

TRACY **ZIMMERMANN, PHD**
Chief Development Officer

JENNIFER **ELLIOTT, PHD, JD**
Chief Legal Officer

ANTOINETTE **PAONE, MS, MBA**
SVP, Head of Regulatory Affairs & Quality

MATT **STANTON, PHD**
Chief Scientific Officer

DOUG **KERR, MD, PHD**
Chief Medical Officer

SARA **DEN BESTEN**
SVP, Human Resources

PHILLIP **SAMAYOA, PHD**
VP, Strategy & Portfolio Development, Founder

ZHONG **ZHONG, PHD**
VP, Head of Gene Therapy

LESLIE **WOLFE, PHD**
SVP, Head of CMC

CASH INTO 2024

TWO LEAD PROGRAMS TO THE CLINIC

DEEP LIVER PORTFOLIO
3rd IND in 2023

EXPANSION TO OTHER TISSUES

*Pro forma as of Jan. 7, 2021

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Platform confirmation sets stage for IND-enabling studies with hemophilia A development candidate

Durability & redosing
in mice

Target levels of factor VIII expression
in Hemophilia A mice

Translation of expression across species
from mouse to NHP

IND-enabling studies
Hemophilia A

Demonstrated durability and redosing with Factor IX in immune competent mice

No decrease in expression throughout study period, redosing increases expression proportionately

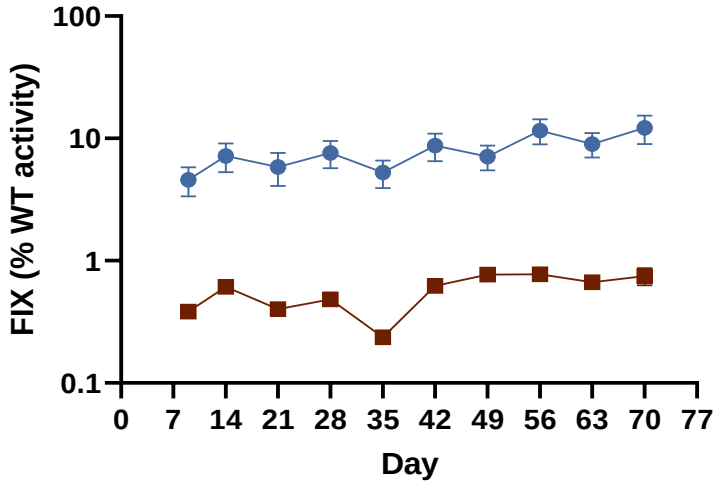
Durability & redosing *in mice*

Target levels of factor VIII expression *in Hemophilia A mice*

Translation of expression across species *from mouse to NHP*

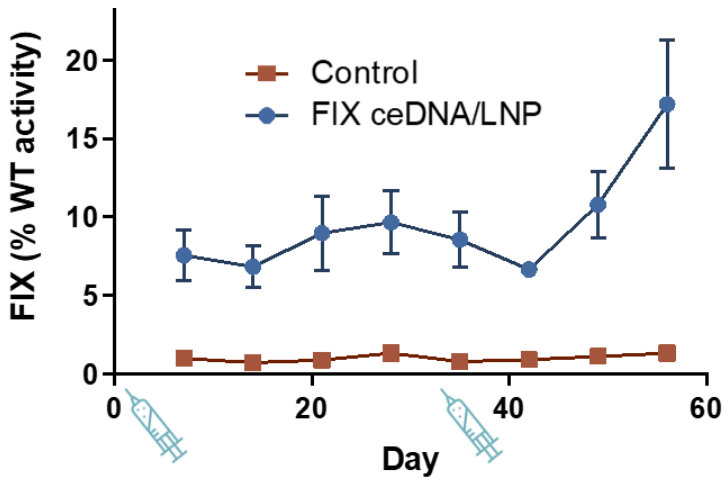
IND-enabling studies *Hemophilia A*

Factor IX



- Single IV administration at study day 0
- Factor IX activity calculated from protein ELISA

Factor IX



- Single IV administration at study day 0
- Re-dosed at day 36 at same dose level
- Factor IX activity calculated from protein ELISA

Target levels of factor VIII expression achieved in hemophilia A mice

Durability & redosing *in mice*

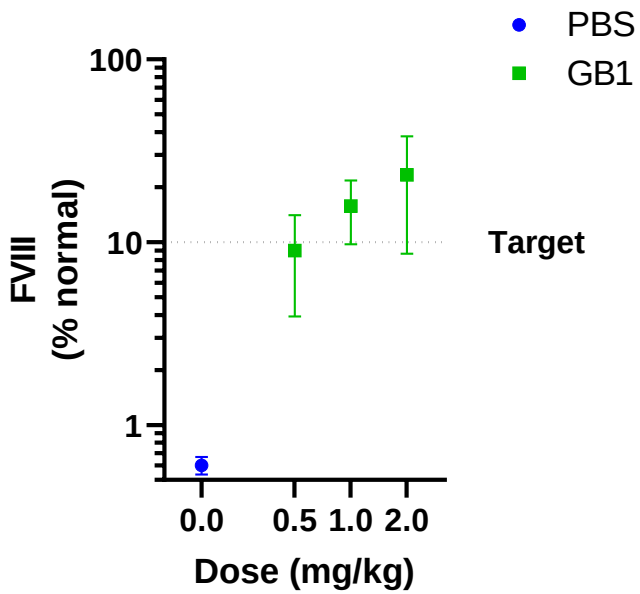
Target levels of factor VIII expression *in Hemophilia A mice*

Translation of expression across species *from mouse to NHP*

IND-enabling studies *Hemophilia A*

Development Construct: FVIII Expression

Systemic IV administration via ctLNP (Day 10)



Key Takeaways

- Significant mean human factor VIII expression
  - *23% of normal at 2 mg/kg*
  - *16% of normal at 1 mg/kg*
  - *9% of normal at 0.5 mg/kg*
- Clear dose-response relationship demonstrated
- Well tolerated at all dose levels
- In our clinical program, we are targeting an average of 10% factor VIII activity per dose in patients

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Translation of expression observed from mice to non-human primates

Durability & redosing *in mice*

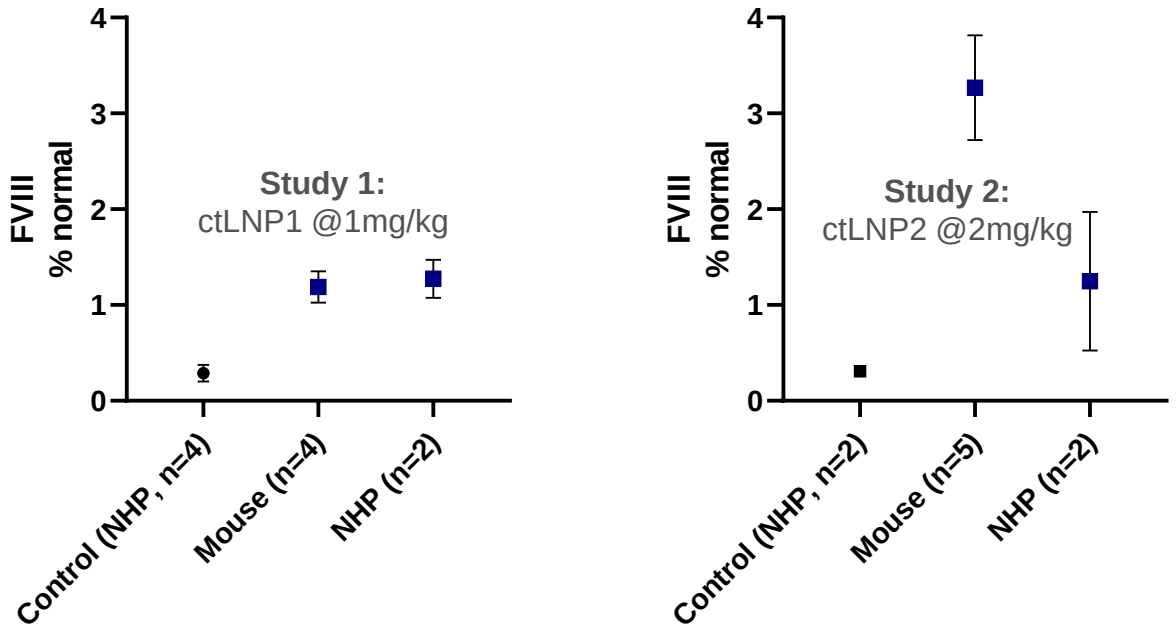
Target levels of factor VIII expression *in Hemophilia A mice*

Translation of expression across species *from mouse to NHP*

IND-enabling studies *Hemophilia A*

Research Construct: Species Translation

Systemic IV administration via ctLNP (day 5 or 7)



Key Takeaways

- Two studies conducted with similar ctLNPs at doses of 1-2 mg/kg
- Observed translation of approximately 2:1 from mouse to NHP using weight-based dosing
- Well tolerated in NHP up to 2 mg/kg, the highest dose tested
- Consistent with validated LNPs

Platform confirmation sets stage for IND-enabling studies with hemophilia A development candidate; on track for IND in 2022

Durability & redosing *in mice*

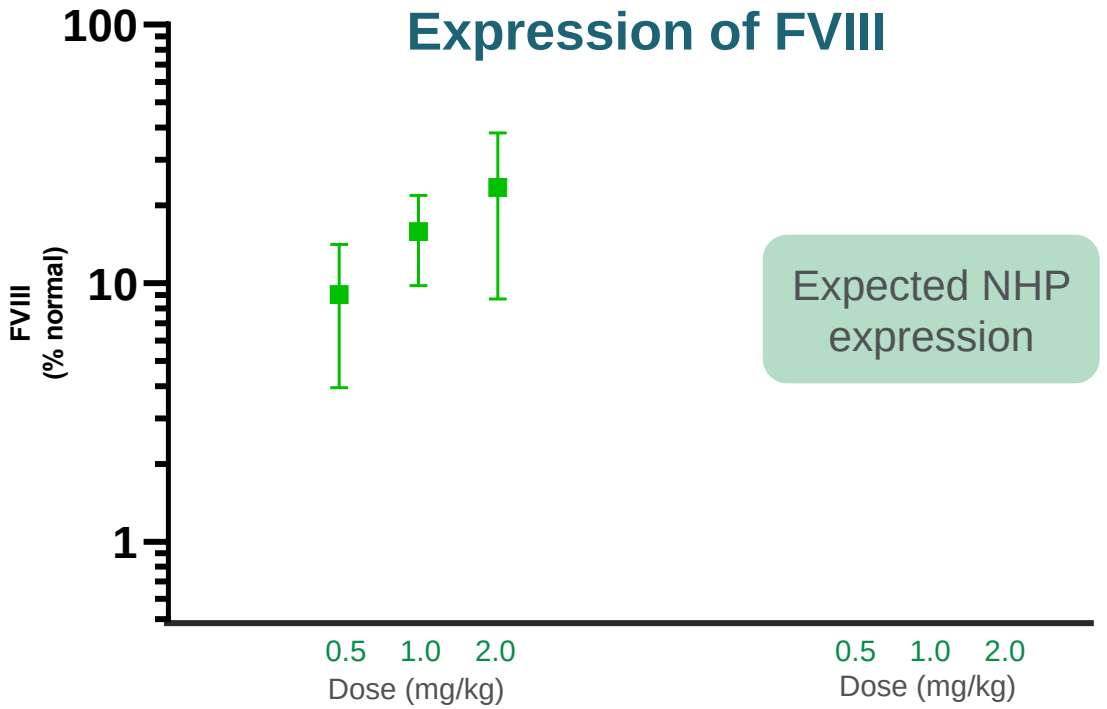
Target levels of factor VIII expression *in Hemophilia A mice*

Translation of expression across species *from mouse to NHP*

IND-enabling studies *Hemophilia A*

Next Steps

- Selection of hemophilia A development candidate
- NHP Factor VIII expression with development candidate
- Pre-IND meeting with FDA



Our strategy: rapid expansion within areas following proof of concept

Parallel development planned across a range of areas

- LIVER
- SECRETED PROTEINS
- RETINA
- MUSCLE
- ONCOLOGY
- CNS

Rapid expansion to new rare and prevalent indications following early human proof of concept (hPoC)

PoC

FOLLOWED BY EXPANSION TO

Heme A	PoC	
Gaucher	Rare Disease	
Pompe	Rare Disease	
HAE	Rare Disease	
CoV-2	Prevalent Disease	
HIV	Prevalent Disease	

8-week research cycle

Expected 2021 milestones to expand preclinical programs in liver and beyond

Advancing lead liver programs

- Pre-IND development for heme A, including NHP expression for DC
- Re-dosing in NHP (with Factor IX)
- Pre-IND development for PKU

Additional rare & prevalent liver indications

- Pre-clinical data for follow-on rare indications Gaucher, Wilson disease
- Pre-clinical demonstration of liver expression of therapeutic antibodies

Expanding into other tissue areas

Developing delivery for:

- *Retina*
- *Skeletal Muscle*
- *Oncology*
- *CNS*

Capsid-free manufacturing

Continued scaling of established manufacturing process to enable INDs and broad clinical pipeline

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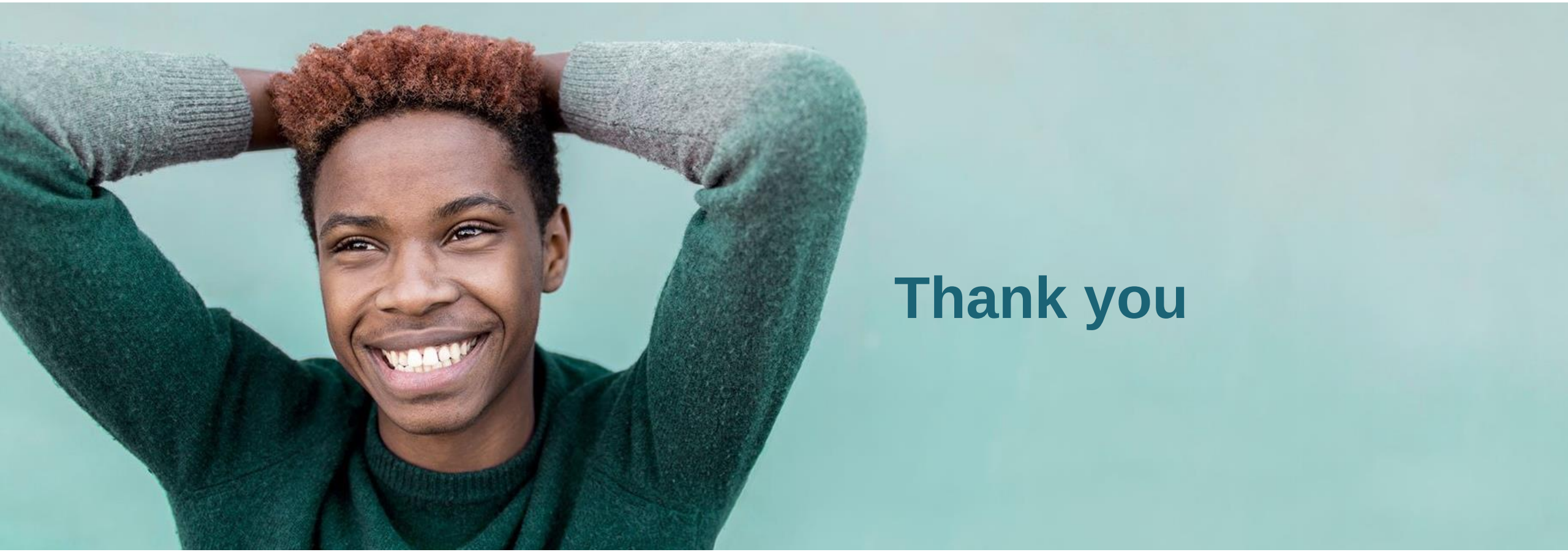

Thank you