

A black and white photograph of a woman with glasses and a lab coat, smiling and holding a pipette. The background is a blurred laboratory setting.

We're pushing the limits of genetic medicine

And our goal is no limits

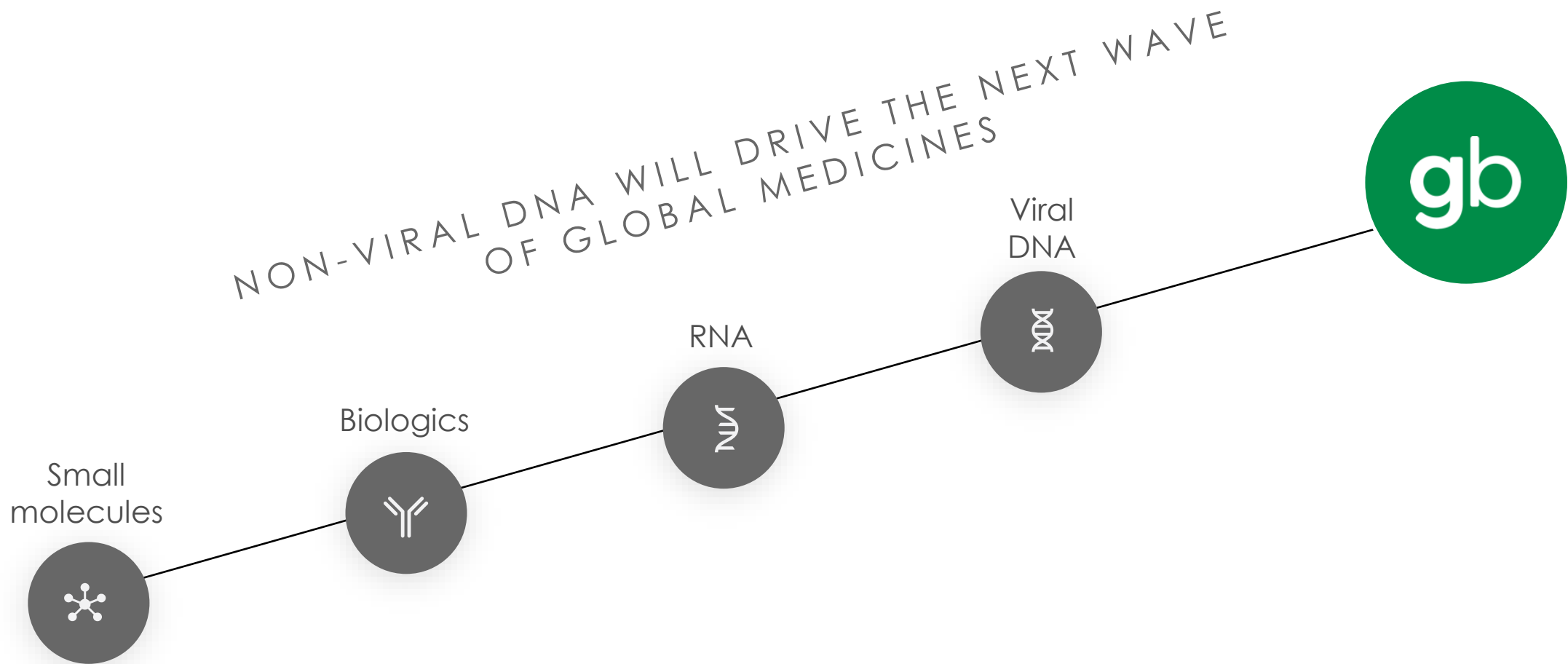
August 2023

generation **bio**[™]

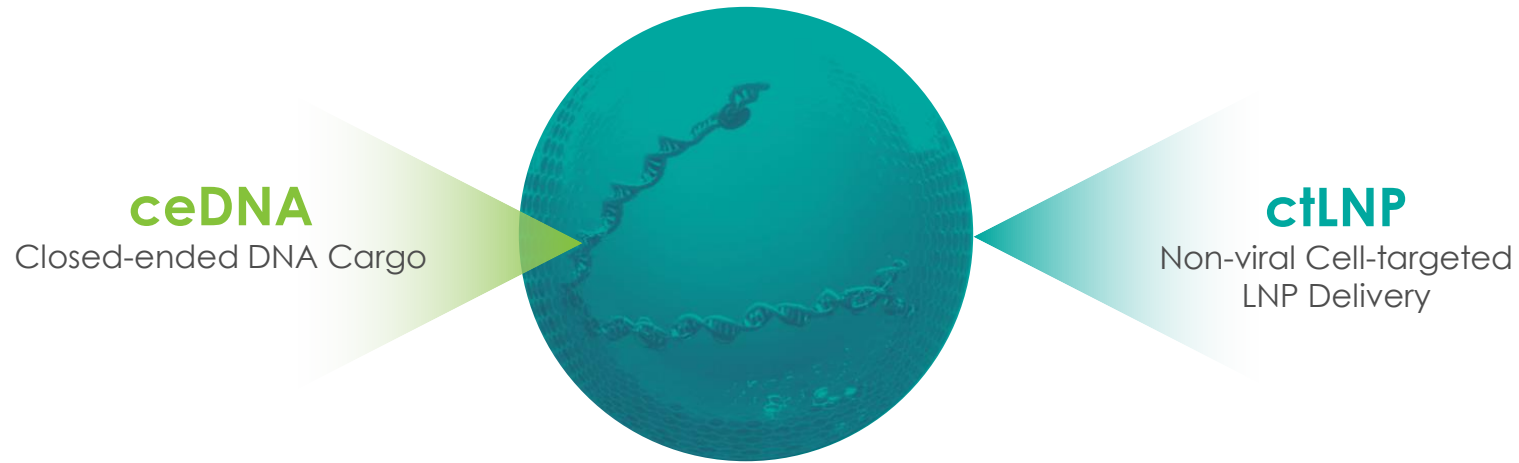
Forward Looking Statements

Any statements in this presentation about future expectations, plans and prospects for the company, including statements about our strategic plans or objectives, our technology platform, including our rapid enzymatic synthesis (RES) technology, our research and clinical development plans, and our preclinical data 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 will be predictive of the results of later preclinical studies and clinical trials; uncertainties regarding the RES manufacturing process; expectations for regulatory approvals to conduct trials or to market products; challenges in the manufacture of genetic medicine products; whether the company’s cash resources are sufficient to fund the company’s operating expenses and capital expenditure requirements for the period anticipated; 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 our most recent annual report on Form 10-K and quarterly report on Form 10-Q, which are 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.

We are the new modality leader for non-viral DNA therapeutics



Unique DNA and delivery make genetic medicines more drug-like



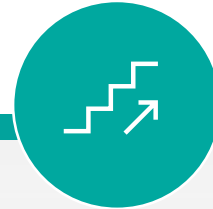
DURABLE

Gene expression for the life of the cell



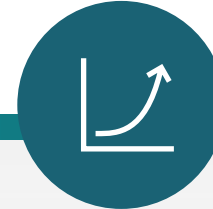
REDOSABLE

Extend therapeutic benefit over the long term



TITRATABLE

Expression levels can be adjusted to meet each patient's need



SCALABLE

Manufacturing to support hundreds of millions of doses globally

Building scale to transform genetic medicines, globally



RES

Rapid enzymatic synthesis for ceDNA

Enables *global scale*

Enables *rapid research cycle*

New modality leader

Leading the rare disease field to durable cures

HEMOPHILIA A

30K
U.S. PATIENTS



Rare disease innovator

Developing a deep rare disease portfolio

RARE LIVER DISEASES

100K
U.S. PATIENTS



Prevalent disease disruptor

Global impact through proprietary & partnered programs

PREVALENT DISEASES

>100M
U.S. PATIENTS



Our focus is on our core liver portfolio, building from rare to prevalent indications

New modality leader

Leading the rare disease field to durable cures

LIVER PIPELINE



- Hemophilia A

Rare disease innovator

Developing a deep rare disease portfolio



- PKU*
- Wilson*
- Gaucher
- Undisclosed*

Prevalent disease disruptor

Global impact through proprietary & partnered programs



- ETAP
- Undisclosed*

Our lead indication is Hemophilia A

LIVER
PIPELINE

New modality leader

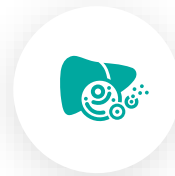
Leading the rare disease field to durable cures



- Hemophilia A

Rare disease innovator

Developing a deep rare disease portfolio



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Global impact through proprietary & partnered programs



- ETAP
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We aim to transform the therapeutic landscape for Hemophilia A

TARGETING A HIGHLY DIFFERENTIATED CLINICAL & COMMERCIAL PROFILE



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



- **Extend genetic medicine to all Hemophilia A patients**
- **Rescue** for undertreated AAV patients

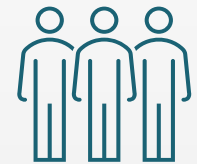


- **More predictable** clinical outcome
- **Reimbursement** in current paradigm

HEMOPHILIA A

- ✓ Clear pre-clinical biomarkers
- ✓ Established regulatory path
- ✓ Significant unmet need
- ✓ Large global market

\$8B+
Global Market



Our unique stealth ctLNP opens unprecedented extra-hepatic franchise opportunities

New modality leader

Leading the rare disease field to durable cures

Rare disease innovator

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LIVER PIPELINE



- Hemophilia A



- PKU*
- Wilson*
- Gaucher
- Undisclosed*



- ETAP
- Undisclosed*

NON-LIVER PORTFOLIO



Ligand optimization



Immune cells
• Undisclosed*



**Skeletal muscle
CNS**

Building ligand technologies through partnership

New modality leader

Leading the rare disease field to durable cures

Rare disease innovator

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LIVER PIPELINE



- Hemophilia A



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NON-LIVER PORTFOLIO



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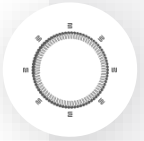


**Skeletal muscle
CNS**

moderna

Stealth ctLNP is basis for immune cell targeting, and for other cell types and tissues

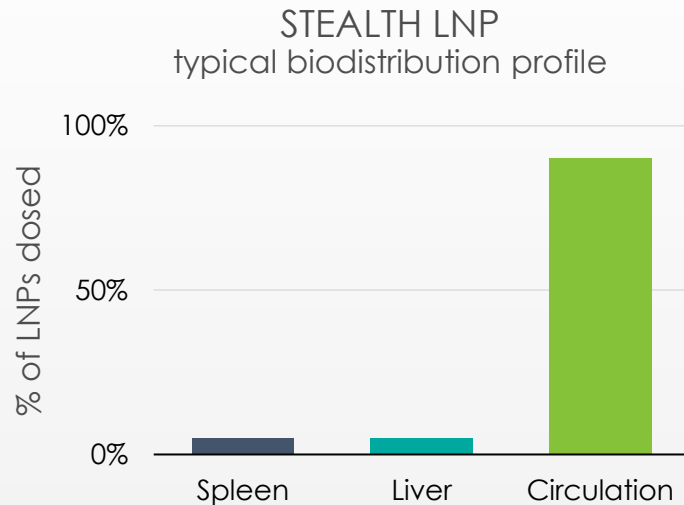
NON-LIVER PORTFOLIO



Ligand optimization

Immune cells

New tissues



MODERNA COLLABORATION

Developing immune cell targeting
for stealth ctLNPs



ONCOLOGY
IMMUNOLOGY
INFECTIOUS DISEASE



NEW CELL TYPES & TISSUES

to create new portfolios of partnered or
wholly-owned programs outside of the liver



GENETIC MYOPATHIES

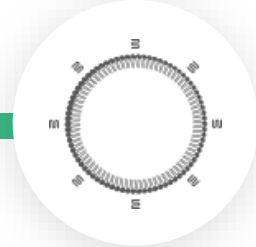


CNS DISEASES

The GBIO platform is designed to create extraordinary leverage



Rapid development of programs within tissues



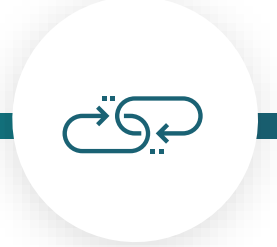
Multiple first-in-class tissue franchises



Global scale for millions of patients



Drug-like profile in development and launch



Redosing paradigm familiar to payors



Thank You

August 2023

generation bio™

