generation bio™

Leading **durable redosable scalable** non-viral genetic medicines

FOR MILLIONS OF PATIENTS LIVING WITH RARE AND PREVALENT DISEASES

NASDAQ: GBIO

JANUARY 2022

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, 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 timing and ability to complete the build-out of the company’s manufacturing facility and regarding the new 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.

Generation Bio is unlocking the full potential of non-viral genetic medicines

Novel platform enables unique clinical profile…

- **DURABLE**
- **REDOSABLE**
- **SCALABLE**

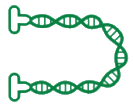
…enabling access to a broad set of large therapeutic areas

- **MULTIPLE MODALITIES**
- **GLOBAL SCALE**
- **MULTIPLE TISSUES**

Our proprietary non-viral genetic medicine platform

THREE CORE PLATFORM TECHNOLOGIES

Multiple Modalities



ceDNA
CLOSED-ENDED DNA

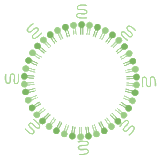
29 PATENT FAMILIES

Global Scale

MFG
INTERNAL LARGE SCALE MANUFACTURING CAPACITY

3 PATENT FAMILIES & TRADE SECRETS

Multiple Tissues



ctLNP
CELL-TARGETED LNP DELIVERY

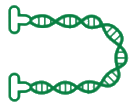
15 PATENT FAMILIES

Patent family numbers current as of January 3, 2022

ceDNA – one construct, multiple modalities

ENABLES ACCESS TO BROAD THERAPEUTIC MECHANISMS

Multiple Modalities



ceDNA
CLOSED-ENDED DNA

29 PATENT FAMILIES

Gene transfer

$22.4bn
total mkt cap

Durably express full transgene

Therapeutic antibodies

$47.2bn
total mkt cap

Durably express and secrete full mAb

Vaccines

$142.5bn
total mkt cap

Express target antigen(s)

Gene editing

$29.6bn
total mkt cap

DNA template for genomic insertion/correction

Patent family numbers current as of January 3, 2022
Market cap data as of January 6, 2022

Rapid Enzymatic Synthesis - building internal capacity for rare and prevalent diseases

MFG TO MATCH SCALE, BREADTH OF PLATFORM POTENTIAL

Global Scale

MFG

INVESTING IN LARGE SCALE INTERNAL MANUFACTURING

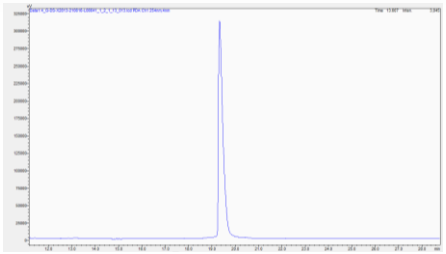
3 PATENT FAMILIES & TRADE SECRETS

Internal cGMP Capacity

104,000 sq. ft. facility in Waltham, MA

- State-of-the art facility to enable clinical and initial commercial supply for multiple potential launches

Quality



IEX chromatography demonstrating high purity

- Consistently yields highly pure ceDNA

Speed

28-day biologic production cycle

shortened to…

1-day enzymatic process

ENABLING

4-week research cycle

- Accelerates preclinical research and development

Patent family numbers current as of January 3, 2022

ctLNP – each tissue-specific ctLNP creates modular access to a therapeutic area

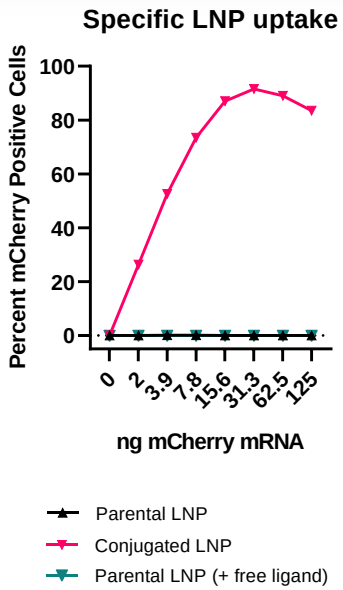
LEADING PORTFOLIO OF ctLNP EXPERTISE AND ASSETS

Multiple Tissues

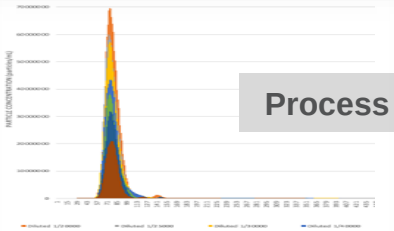
ctLNP
CELL-TARGETED
LNP DELIVERY

15 PATENT FAMILIES

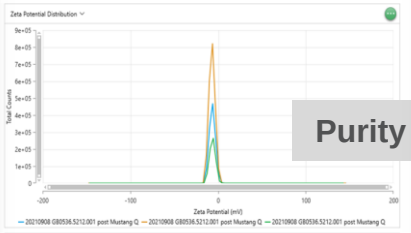
Targeting



Formulation Expertise



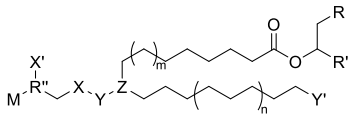
70-75 nm diam.
Polydispersity <0.05



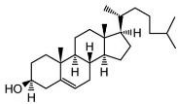
Uniform zeta,
99% encapsulation

Proprietary Lipids

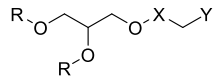
Ionizable Lipids
Several Classes



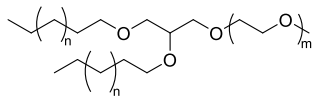
Cholesterol & analogs



Helper lipids



Stabilizing polymer lipids



Modular Access

- LIVER
- RETINA
- VACCINES
- MUSCLE
- TUMOR
- CNS

Patent family numbers current as of January 3, 2022

generation bio

Broad portfolio of rare and prevalent indications in the liver and retina, enabled by modular ctLNP delivery

CORE AREAS OF DEVELOPMENT

TISSUE	PREVALENCE	PROGRAM	RESEARCH	LEAD OPTIMIZATION	PRE-CLINICAL DEVELOPMENT
Liver	Rare	Hemophilia A			
		PKU			
		Wilson Disease			
		Gaucher Disease			
	Prevalent	ETAP*			
Retina	Rare	Stargardt			
		LCA10			
	Prevalent	Wet AMD			

EARLY RESEARCH

- Vaccines

EXPANSION AREAS

- Skeletal Muscle
- Oncology
- CNS

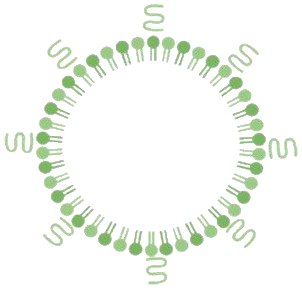
*Endogenous Therapeutic Antibody Production

ctLNP enables modular access to tissues and therapeutic areas

PRIMARY FOCUS

Liver

SYSTEMIC ctLNP DELIVERY



RARE LIVER DISEASES
100K
U.S. PATIENTS

PREVALENT DISEASES
>10M
U.S. PATIENTS

SECONDARY DEVELOPMENT AREAS

Retina

LOCAL ctLNP DELIVERY

RETINAL DISEASES
>1M
U.S. PATIENTS

Vaccine

LOCAL vLNP DELIVERY

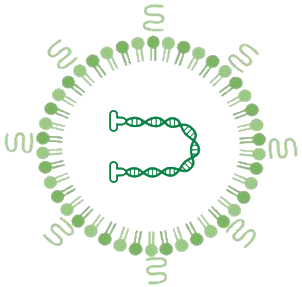
INFECTIOUS DISEASES
>100M
U.S. PATIENTS

ctLNP enables modular access to tissues and therapeutic areas; cargo options

PRIMARY FOCUS

Liver

SYSTEMIC ctLNP DELIVERY



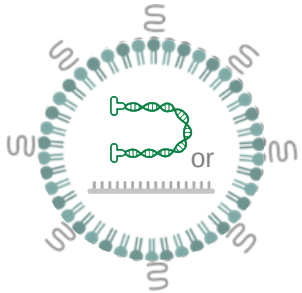
RARE LIVER DISEASES
100K
U.S. PATIENTS

PREVALENT DISEASES
>10M
U.S. PATIENTS

SECONDARY DEVELOPMENT AREAS

Retina

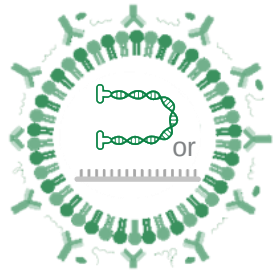
LOCAL ctLNP DELIVERY



RETINAL DISEASES
>1M
U.S. PATIENTS

Vaccine

LOCAL vLNP DELIVERY



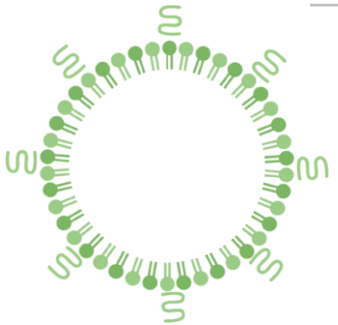
INFECTIOUS DISEASES
>100M
U.S. PATIENTS

ctLNP enables redosable, biologically driven cell and tissue targeting

ctLNP builds on the success of clinically-validated redosable LNP systems

LNP (mRNA)

- 1:1 NHP → human translation
- Biodegradable lipids
- Application expanded from liver to immune cells for vaccines



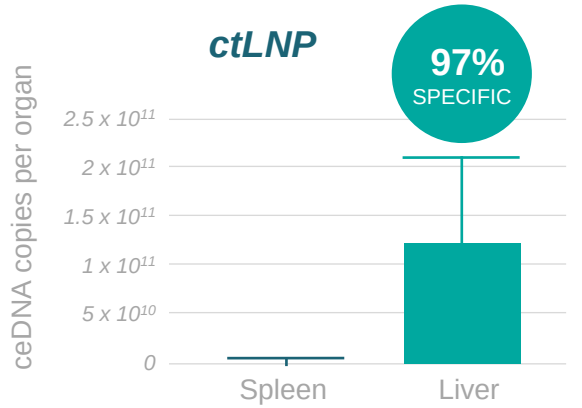
ctLNP

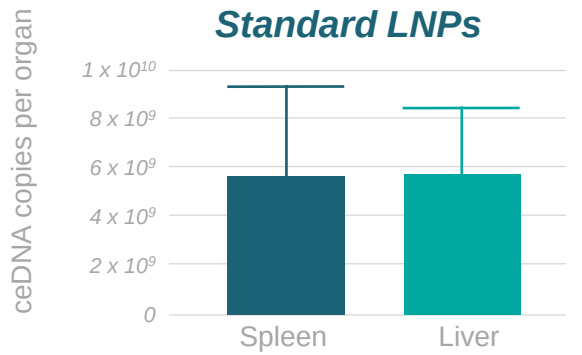
- Actively targeted for cell selectivity
- Minimal off-target distribution
- Applicable to cells/tissues beyond the liver

LIVER
RETINA
VACCINES
MUSCLE
TUMOR
CNS

GalNAc-targeted ctLNP

Highly specific hepatocyte delivery





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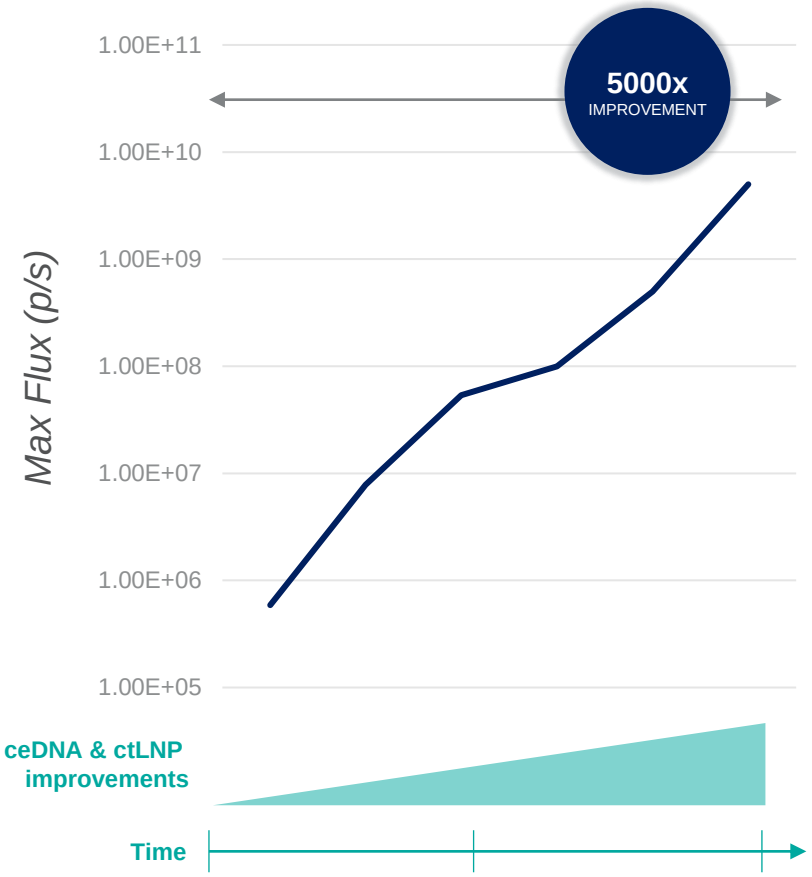
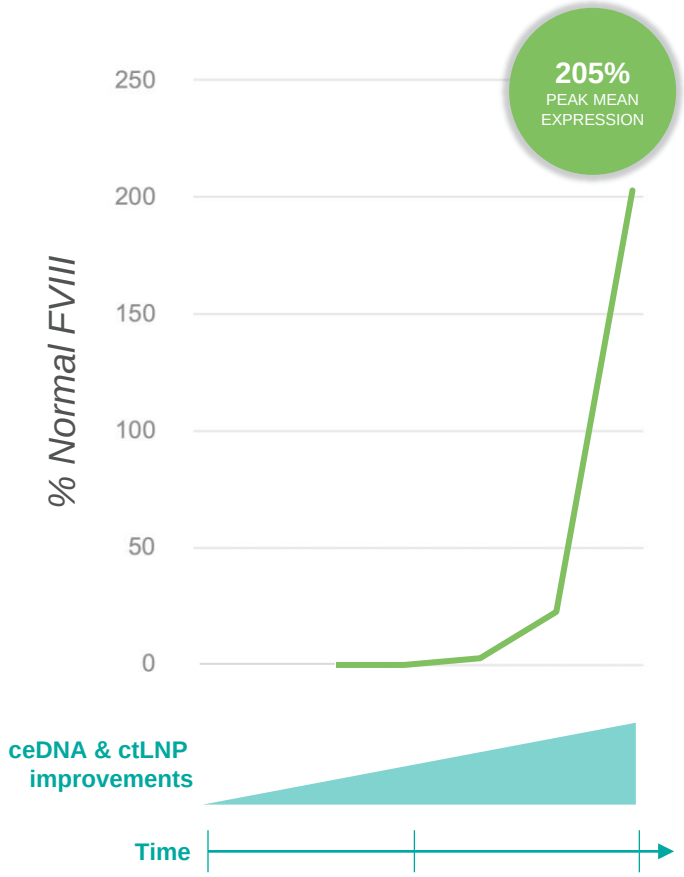
Substantial platform progress with leading profile for non-viral DNA delivery and expression in liver

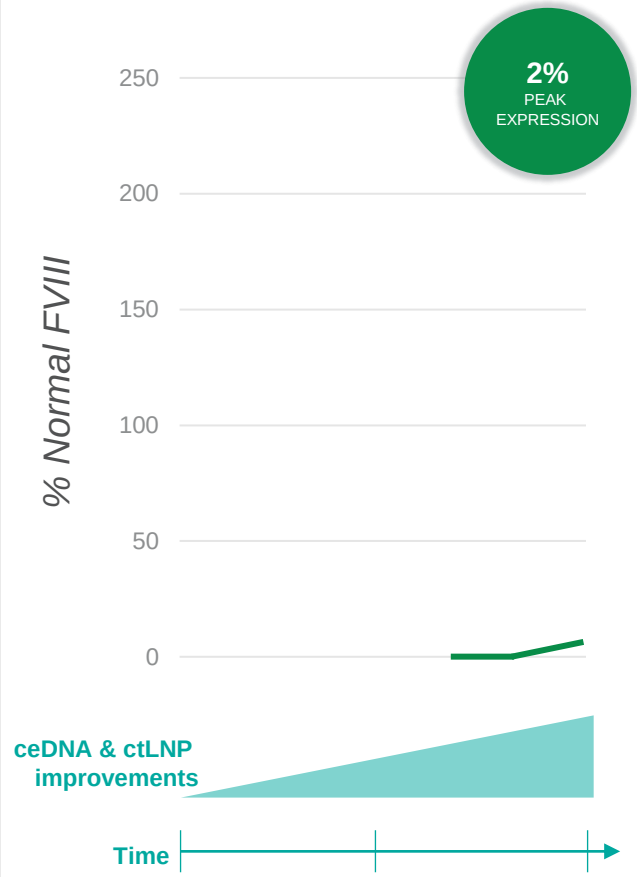
Initial optimization with luciferase in mice



Rapid progress with Factor VIII in mice



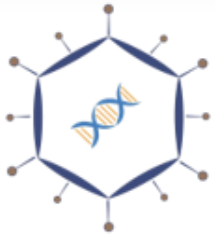
Current translation with Factor VIII in NHP



generation bio

Developing novel ctLNP to enable broad access to photoreceptors and RPE

ctLNP aiming for best-in-class non-viral delivery to the retina

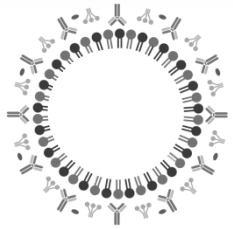


AAV

Current gold standard for retinal gene therapy

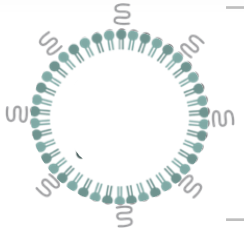
Limitations:

- Cargo size
- May require dual AAV, increasing possibility for off-target tox
- Sub-optimal retina transduction



Standard LNPs

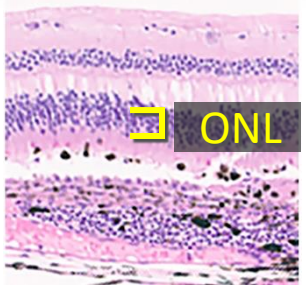
- Theoretically address AAV limitations, but…
- Poor tolerability and severe retinal degeneration
- Low expression



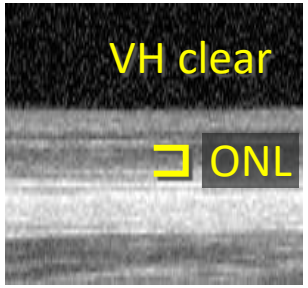
ctLNP

- Minimal distribution to immune cells
- Preservation of photoreceptors in outer nuclear layer (ONL)
- Unique broad retinal transduction

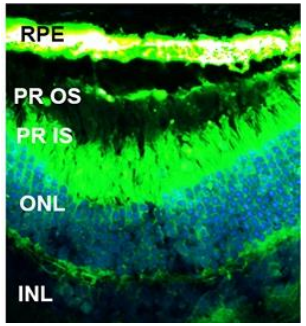
Target subretinal ctLNP profile: stable ONL, broad expression



Tolerability (H&E)



Tolerability (OCT)



Biodistribution (IHC)

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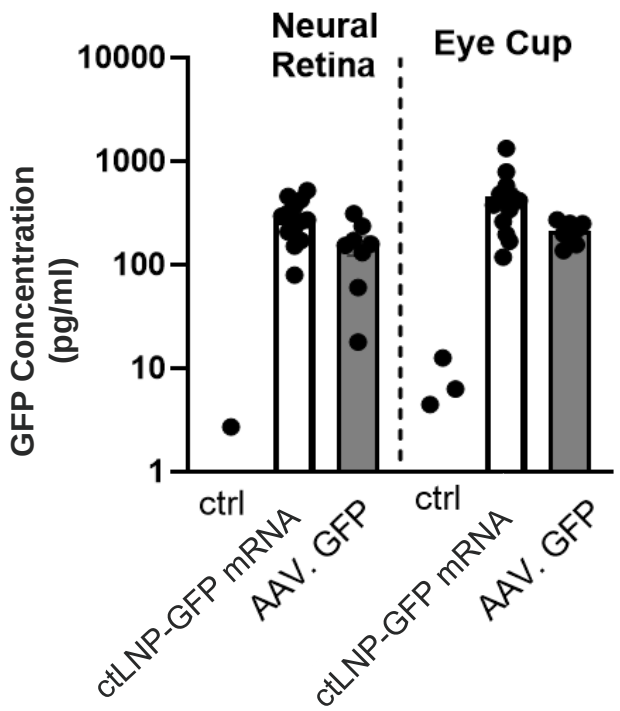
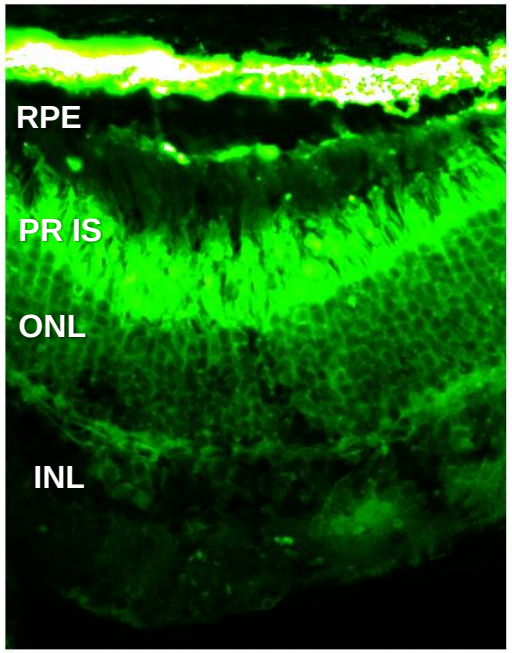
ctLNP-mRNA shows broad photoreceptor distribution versus AAV5 which appears punctate

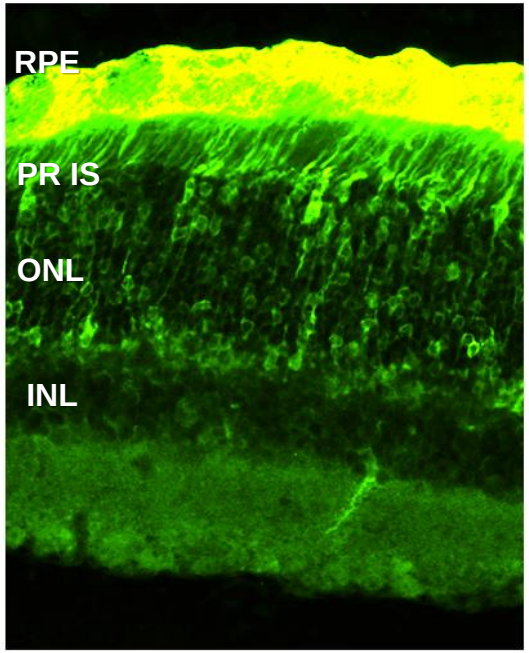
ctLNP-mRNA shows total expression in photoreceptors comparable to AAV5



ctLNP-GFP mRNA at 24 h



AAV5.GFP at Day 28



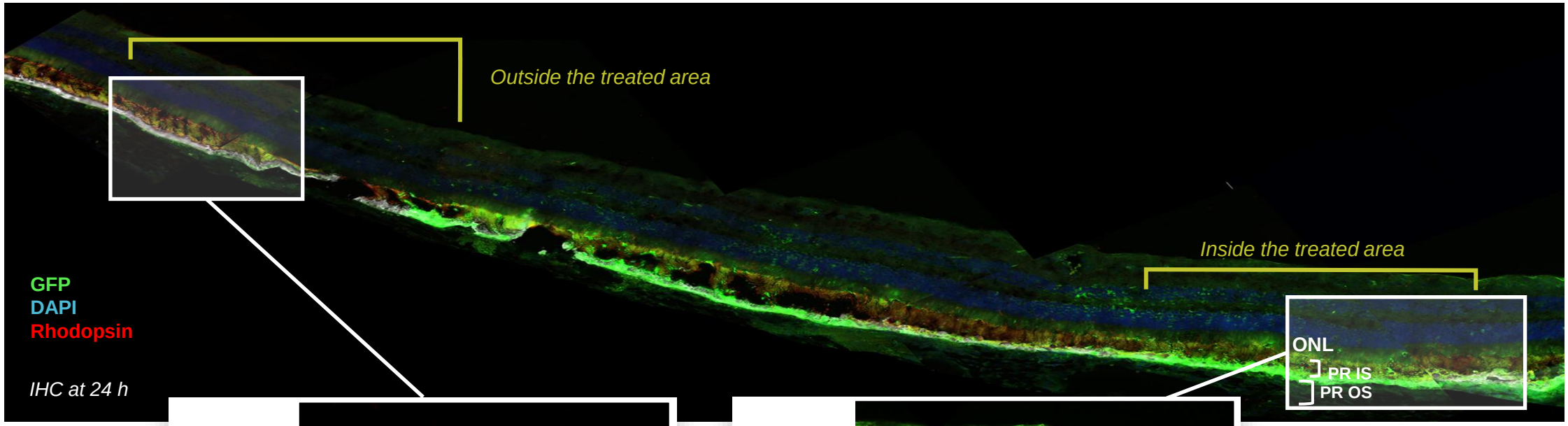
Expression data from mouse

Data presented at ESGCT 2021

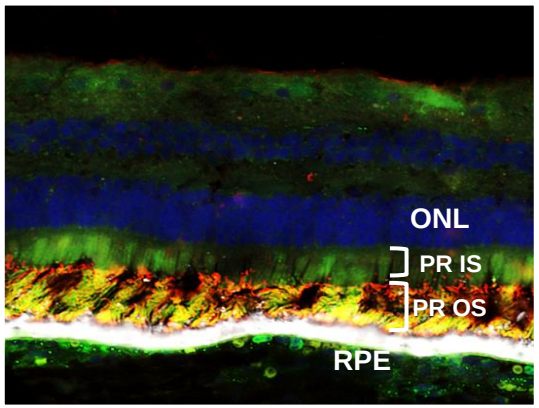
generation bio

ctLNP-GFP mRNA transduces NHP photoreceptors and RPE

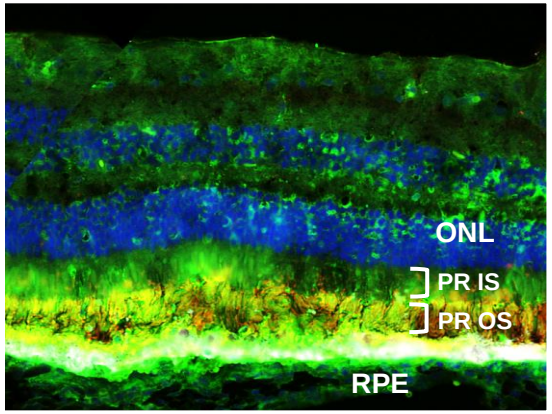
Preserved photoreceptor layer (ONL) and broad GFP expression in treated area (SR injection)



Outside the SR bleb



Within the SR bleb



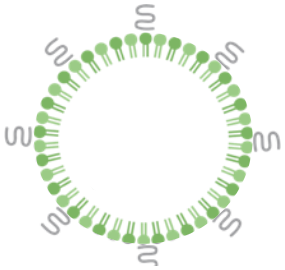
Data presented at ESGCT 2021

generation bio

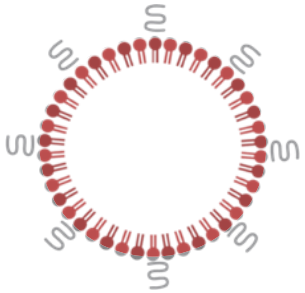
ctLNP optimization shows improved BioD and potency in photoreceptors and RPE

Optimization of ctLNP composition

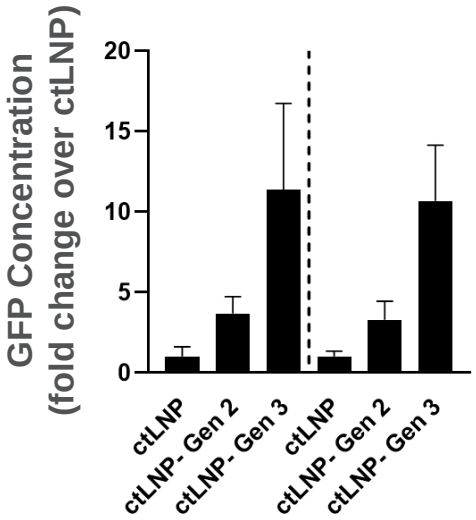
ctLNP



ctLNP- Gen 2



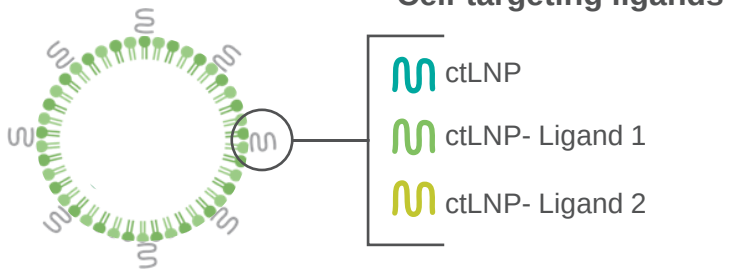
ctLNP- Gen 3

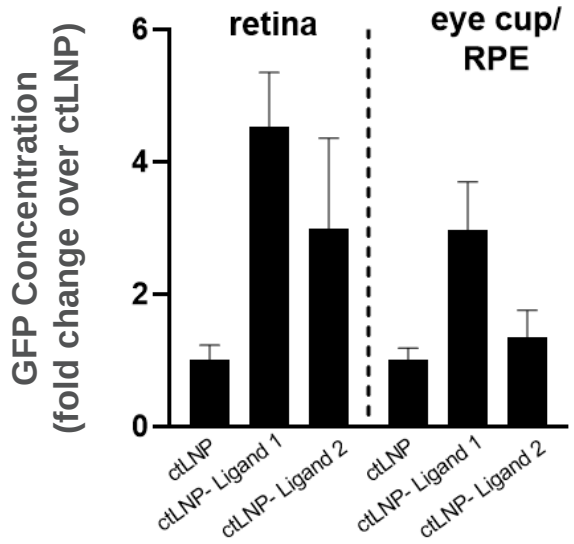


ctLNP- Gen 3

In vivo mouse expression
ctLNP-GFP mRNA

Optimization of ctLNP targeting ligands





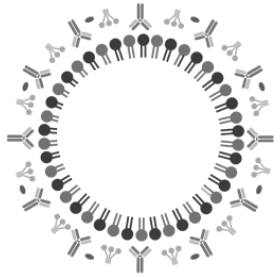
In vivo mouse expression
ctLNP-GFP mRNA

Data presented at ESGCT 2021

generation bio

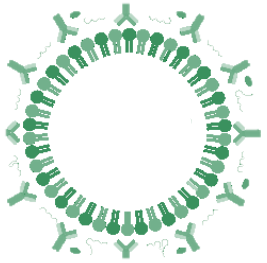
Expanding our LNP technology from cell-targeted to vaccine-optimized compositions

Novel proprietary LNPs tailored to nucleic acid vaccines



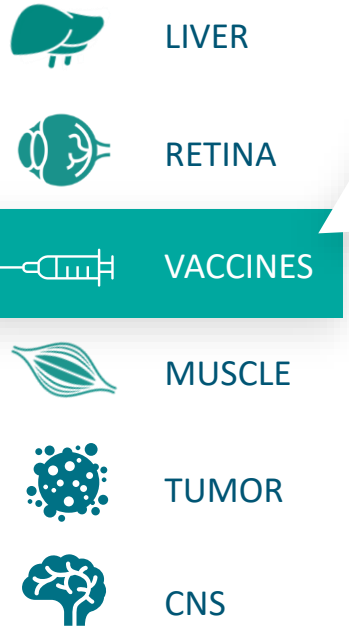
LNP (mRNA)

- 1:1 NHP → human translation
- Biodegradable lipids
- Application expanded from liver to immune cells for vaccines

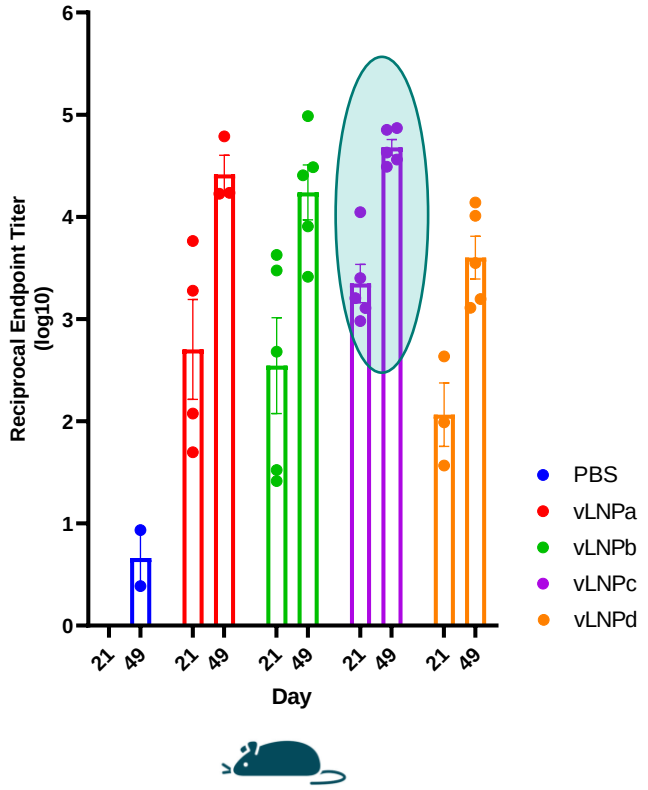


vLNP

- Proprietary vaccine optimized LNP (vLNP)
- Optimized for immune cell distribution and expression
- Utilizes proprietary lipid estate



Screening of LNPs to directly identify those with optimal immunogenicity profile



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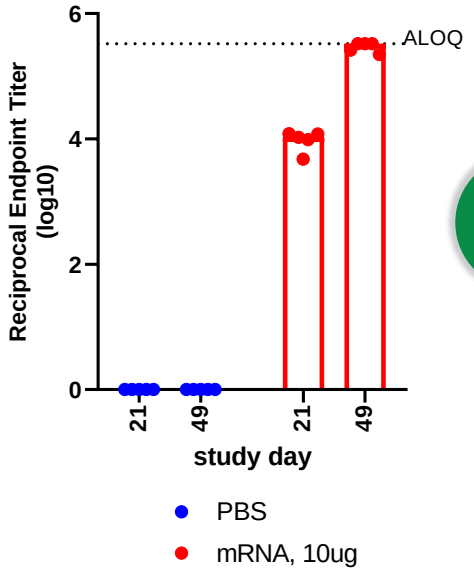
vLNP-mRNA achieves benchmark antibody levels and neutralization across species

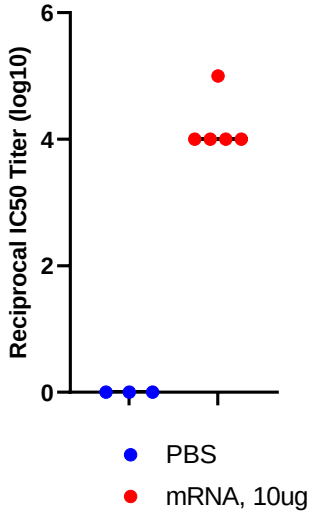
Strong neutralizing antibody response

Anti-CoV-2 Spike binding Abs (mice)



10µg DOSE

Pseudotype virus (CoV-2) neutralization

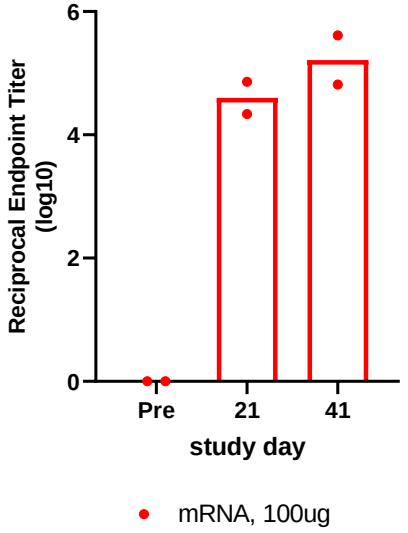


Prime day 0, boost day 28

vLNP-mRNA benchmark on par with Moderna's mRNA-1273 in Balb/c mice (Corbett Nature 2020)

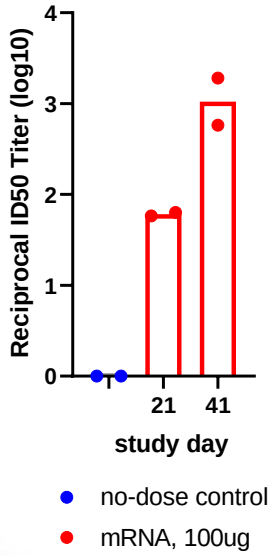
Consistent species translation to NHP

Anti-CoV-2 Spike binding Abs (NHP)



100µg DOSE

Live virus (CoV-2) neutralization



Prime day 0, boost day 28

vLNP-mRNA benchmark on par with Moderna's mRNA-1273 in cynomolgus monkeys (Corbett NEJM 2020)

Neutralization data generated in collaboration with the Douam Lab at Boston University

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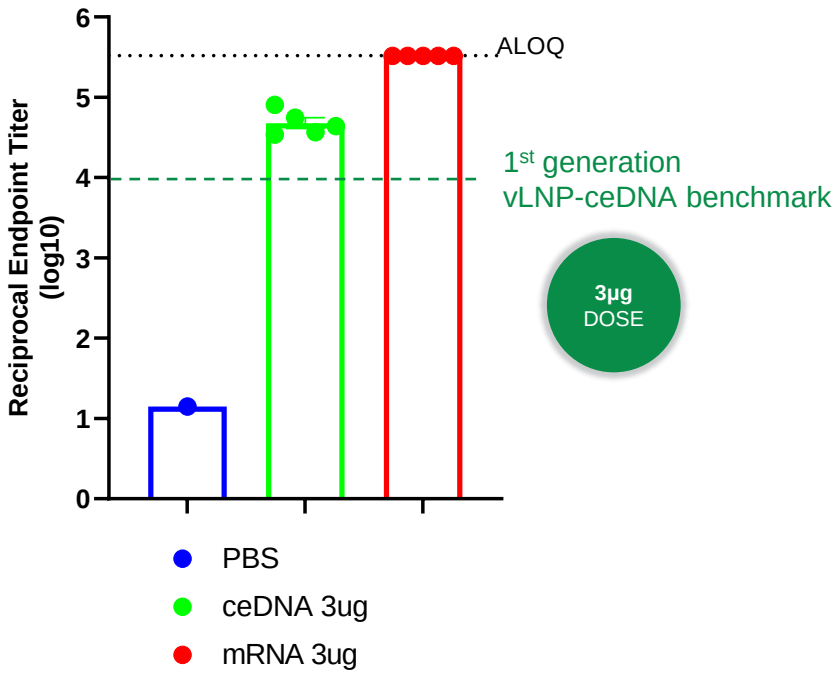
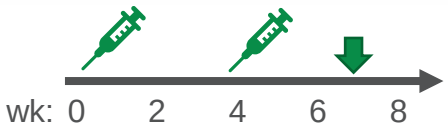
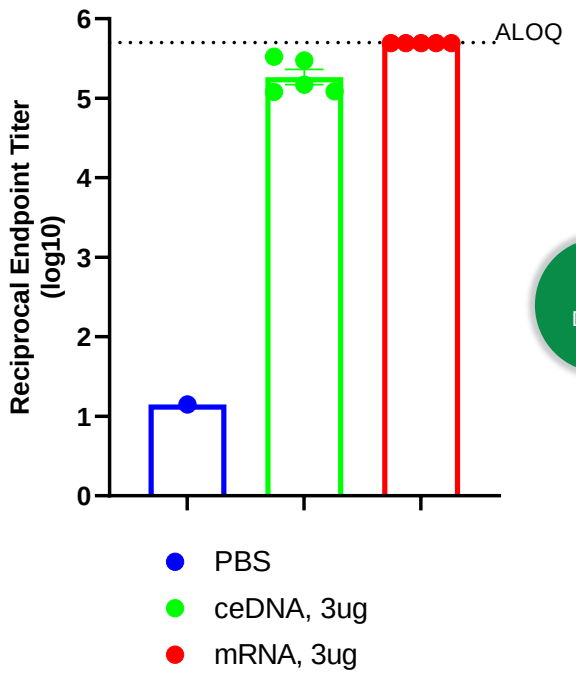
vLNP optimization further increases vaccine potency

Next-generation vLNP-ceDNA demonstrates enhanced antibody response

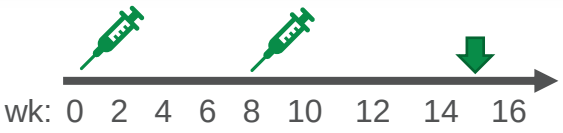




Antibody response further enhanced by longer prime-boost spacing



- High antibody binding titers are maintained 7 weeks post-boost



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World class team and strong balance sheet

PROVEN TEAM TRACK RECORD
40+ IND filings, 20 approved products

$400M CASH BALANCE*
to fund innovation

RUNWAY
Into 2024

LEADERSHIP TEAM

GEOFFREY
MCDONOUGH, MD
President & CEO
sobi · genzyme A SANOFI COMPANY

MATTHEW
NORKUNAS, MD, MBA
Chief Financial Officer
SomaLogic · MARSICO CAPITAL MANAGEMENT, LLC

MATT
STANTON, PHD
Chief Scientific Officer
moderna · MERCK

JENNIFER
ELLIOTT, PHD, JD
Chief Legal Officer
BROAD INSTITUTE · Genentech

DOUG
KERR, MD, PHD
Chief Medical Officer
Biogen · Shire

TRACY
ZIMMERMANN, PHD
Chief Development Officer
Alnylam PHARMACEUTICALS

ANTOINETTE
PAONE
SVP, Regulatory Affairs & Quality
VERTEX · MERCK · MILLENNIUM

ZHONG
ZHONG, PHD
SVP, DNA Sciences
VERTEX · gsk · J&J

SARA
DEN BESTEN
Chief People Officer
genzyme A SANOFI COMPANY · NOVAZYME Pharmaceuticals, Inc. · DEN BESTEN GROUP

PHILLIP
SAMAYOA, PHD
SVP, Head of Corporate Development
ATLAS VENTURE

LESLIE
WOLFE, PHD
SVP, Head of CMC
genzyme A SANOFI COMPANY

SONIA
RAZZETI
VP, Quality Assurance
Ironwood PHARMACEUTICALS · Shire

*Approximate cash balance as of 9/30/21

Generation Bio is unlocking the full potential of non-viral genetic medicines

Multiple Modalities

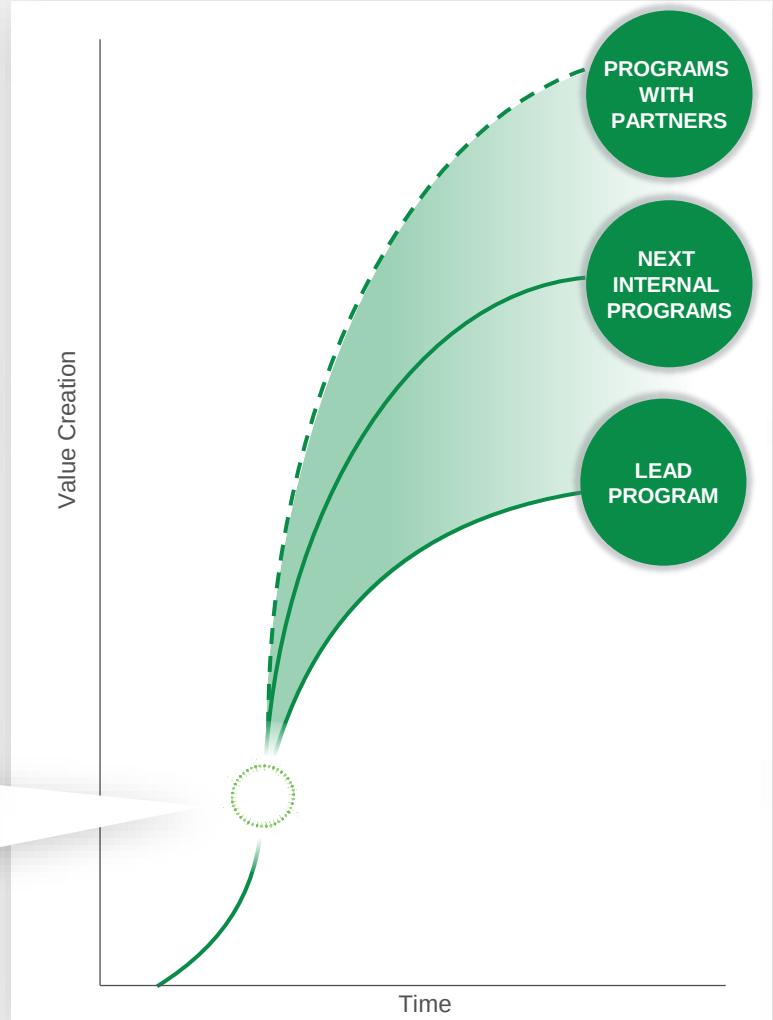
ceDNA
CLOSED-ENDED DNA

Global Scale

MFG
INTERNAL LARGE SCALE MANUFACTURING CAPACITY

Multiple Tissues

ctLNP
CELL-TARGETED LNP DELIVERY



Liver

RARE LIVER DISEASES
100K
PATIENTS

PREVALENT DISEASES
10M
PATIENTS

Retina

RETINAL DISEASES
OVER **1M**
PATIENTS

Vaccine

INFECTIOUS DISEASES
OVER **100M**
PATIENTS

OUR FOCUS

- Finalizing ctLNP for development of liver and retina indications
- Building on vLNP-mRNA vaccine benchmark, develop additional optionality for use of ceDNA in vaccines
- Investing in internal cGMP manufacturing facility
- Leading development of genetic medicines for rare and prevalent diseases in large therapeutic areas

generation bio™

Thank you

JANUARY 2022