



**PRECISION**  
BIOSCIENCES

Dedicated to Improving Life.

January 2020

Overcome cancer.  
Cure genetic disease.  
Feed the planet.

DTIL



# Forward Looking Statements



This presentation (together with any other statements or information that we may make in connection herewith) may contain forward-looking statements. All statements other than statements of present and historical facts contained in this prospectus, including without limitation, statements regarding our future results of operations and financial position, business strategy and approach, including related results, prospective products, planned preclinical or greenhouse studies and clinical or field trials, capabilities, including expected production levels, of our manufacturing facility, management's expectations regarding pipelines and milestones for product candidates and our food editing platform, and timing and likelihood of success, as well as plans and objectives of management for future operations, may be forward-looking statements. Without limiting the foregoing, the words "anticipate," "believe," "could," "expect," "should," "plan," "intend," "estimate," "target," "may," "will," "would," "potential," the negative thereof and similar words and expressions are intended to identify forward-looking statements. These forward-looking statements reflect various assumptions of Precision's management that may or may not prove to be correct. No forward-looking statement is a guarantee of future results, performance, or achievements, and one should avoid placing undue reliance on such statements.

Forward-looking statements are based on our management's beliefs and assumptions and on information currently available to us. Such statements are subject to a number of known and unknown risks, uncertainties and assumptions, and actual results may differ materially from those expressed or implied in the forward-looking statements due to various factors, including, but not limited to, our ability to become profitable; our ability to procure sufficient funding; our limited operating history; our ability to identify, develop and commercialize our product candidates; our dependence on our ARCUS technology; the initiation, cost, timing, progress and results of research and development activities, preclinical or greenhouse studies and clinical or field trials; our or our collaborators' ability to identify, develop and commercialize product candidates; our or our collaborators' ability to advance product candidates into, and successfully complete, clinical or field trials; our or our collaborators' ability to obtain and maintain regulatory approval of future product candidates, and any related restrictions, limitations and/or warnings in the label of an approved product candidate; the regulatory landscape that will apply to our and our collaborators' development of product candidates; our ability to achieve our anticipated operating efficiencies as we commence manufacturing operations at our new facility; our ability to obtain and maintain intellectual property protection for our technology and any of our product candidates; the potential for off-target editing or other adverse events, undesirable side effects or unexpected characteristics associated with any of our product candidates; the success of our existing collaboration agreements; our ability to enter into new collaboration arrangements; public perception about genome editing technology and its applications; competition in the genome editing, biopharmaceutical, biotechnology and agricultural biotechnology fields; potential manufacturing problems associated with any of our product candidates; potential liability lawsuits and penalties related to our technology, our product candidates and our current and future relationships with third parties; and other important factors discussed under the caption "Risk Factors" in our Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2019, as such factors may be updated from time to time in our other filings with the SEC, which are accessible on the SEC's website at [www.sec.gov](http://www.sec.gov)

All forward-looking statements speak only as of the date of this presentation, and except as required by applicable law, we do not plan 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.

This presentation may also contain estimates, projections, and/or other information regarding our industry, our business and the markets for certain of our product candidates, including data regarding the estimated size of those markets, and the incidence and prevalence of certain medical conditions. Unless otherwise expressly stated, we obtained this industry, business, market and other data from reports, research surveys, clinical trials, studies and similar data prepared by market research firms and other third parties, from industry, medical and general publications, and from government data and similar sources. Information that is based on estimates, forecasts, projections, market research, or similar methodologies is inherently subject to uncertainties and actual events or circumstances may differ materially from events and circumstances reflected in this information.

# Dedicated To Improving Life



Overcome Cancer.



Cure Genetic Disease.



Feed the Planet.



# Delivering on the Promise of Genome Editing



*World class team of ~200 Precisioneers that includes the **pioneers** in genome editing*



**ARCUS genome editing platform** built for translation with full freedom to operate

*Clinical stage **allogeneic CAR T platform** with validating initial safety and response data*



*Scaled **in-house cGMP manufacturing***



**In vivo gene correction platform** seeking to cure genetic and infectious diseases

*Wholly integrated **food editing platform** focused on human wellness and food security*



# Multiple Key Milestones Delivered in 2019



Dosed first patients in Phase 1/2a trial with our lead CD19 allogeneic CAR T



Opened first US in-house cGMP facility for genome edited allo CAR T cell therapies



IND cleared for second allogeneic CAR T program targeting CD20



IND filed for third allogeneic CAR T program targeting BCMA



Presented first clinical data from PBCAR0191 Phase 1 trial at ASH 2019

# ARCUS: Engineering Nature's Genome Editing System



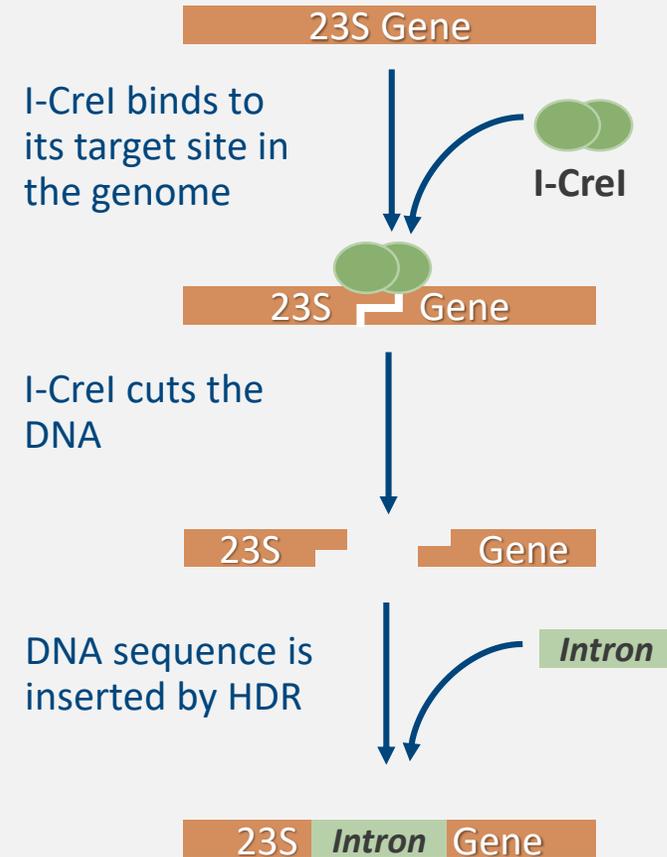
ARCUS is derived from I-Crel, a homing endonuclease naturally evolved for highly precise genome editing

## Four Key Attributes

- 1. Safety:** Evolved to avoid random off-targeting
- 2. Easy to Deliver:** Small size compatible with existing gene delivery technologies
- 3. Control of Edits:** Knock genes *in* or knock genes *out*
- 4. Proprietary:** Complete control of platform and freedom to operate

## Genome Editing by I-Crel in Algae

*Chlamydomonas reinhardtii*



# In-House cGMP Manufacturing for Key Components of Portfolio



- Manufacturing Center for Advanced Therapeutics (MCAT) is a 17,300 square foot cGMP clinical manufacturing facility
- Allogeneic CAR T cells, mRNA (10g scale) and rAAV (400L scale) vectors for *in vivo* and *ex vivo* uses
- Second phase expansion for commercial application (> 10,000 CAR T doses / treatments per year)
- **cGMP BCMA drug product will be produced at this facility**



# Deep Bench of Talent Across All Core Activities



## Senior Leadership

Includes recognized **global pioneers in genome editing**

## Team

Over **200 Precisioneers** including more than 85 PhD scientists and engineers

## Core Competencies

Translational research, development, CAR T / AAV / mRNA manufacturing, clinical trial execution

## Domain Expertise

Across genetics, biochemistry, protein engineering, immunology, clinical oncology, rare diseases, food & agriculture

## Thought Leadership

Management supported by board of directors comprising experts in healthcare research, operations and investment; highly engaged Scientific Advisory Board of **global thought leaders in oncology, CAR T and genetics**



# Curing Genetic Disease

## *In Vivo* Gene Correction



# Why ARCUS for *In Vivo* Gene Correction?



1

## Safety

Evolved to avoid random off-targeting, a critical parameter for safety

2

## Delivery

ARCUS "fits" in established gene delivery vehicles like AAV

3

## Control of Edits

ARCUS enables gene addition, gene knockout, gene repair

4

## Proprietary

ARCUS is the "drug" so freedom-to-operate and IP protection are critical

# In Vivo Gene Correction Pipeline



Product Candidate	Program Area	Discovery	Pre-clinical	Clinical	Rights
HBV	Chronic Hepatitis B – IND 2021				GILEAD
PCSK9	Familial hypercholesterolemia				
Transthyretin	Familial amyloid polyneuropathy				
ApoC3	Lipoprotein lipase deficiency				
HAO1	Primary hyperoxaluria				

# Pioneering Liver Editing Collaboration with University of Pennsylvania



A collaboration with Jim Wilson and the Penn Orphan Disease Center was initiated August, 2016 to develop *in vivo* gene editing in the liver



## Scope

- **Indications:**
  - Familial hypercholesterolemia (*PCSK9*)
  - Transthyretin amyloidosis (*TTR*)
  - Lipoprotein lipase deficiency (*ApoC3*)
  - Primary hyperoxaluria type 1 (*HAO1*)
- **56** non-human primates treated
- **9** unique ARCUS nucleases evaluated
- **Delivery:** AAV and LNP/mRNA delivery

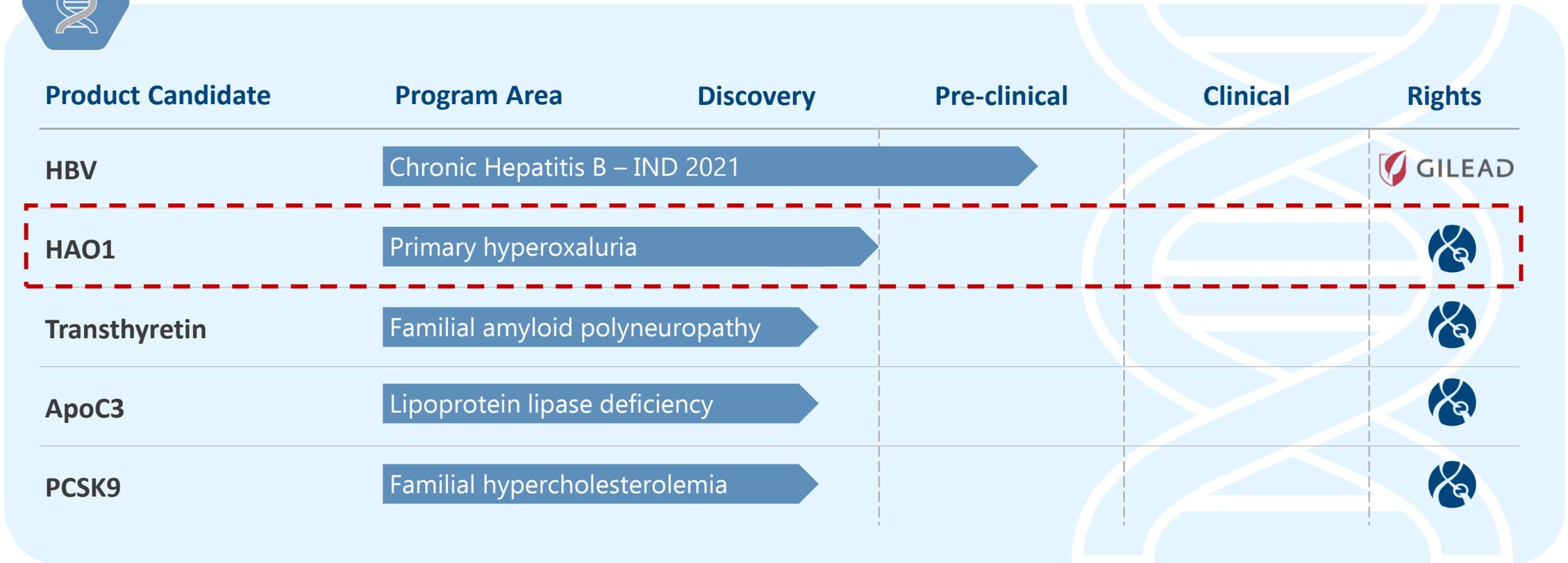
## Parameters

- **Editing efficiency and durability**
- **Toxicity** both acute and long-term
- **Immunogenicity** pre-existing or acquired
- **Off-target editing**
- **Persistence** of ARCUS and vector

**First published report of *in vivo* gene editing in a large animal**

(*Nature Biotechnology* 2018. 36(8):717-725)

# In Vivo Gene Correction Pipeline

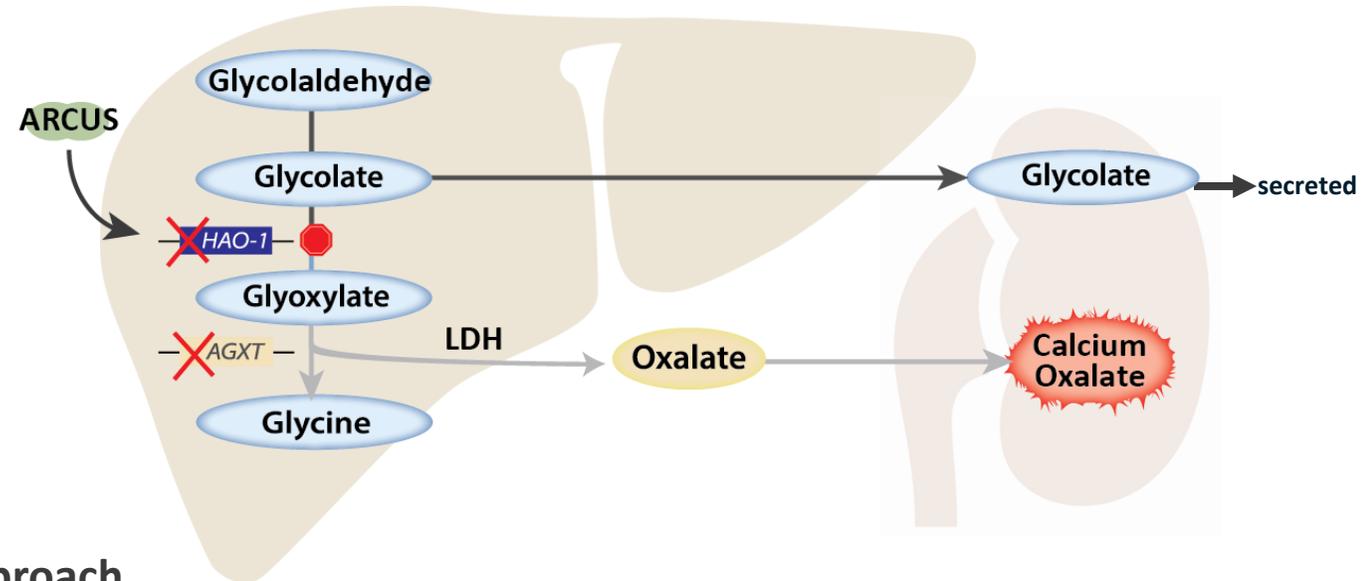


**HAO1 nominated as lead wholly-owned *in vivo* program**

# Primary Hyperoxaluria Type 1 (PH1) – Disease Overview



- 1 Rare genetic disease caused by loss-of-function mutations in the *AGXT* gene**
  - Gene encodes an enzyme involved in glycine biosynthesis in the liver
  - Disease prevalence: 1-3/1,000,000
- 2 Gene mutations lead to the accumulation of calcium oxalate crystals in the kidneys**
  - Causes painful and potentially fatal kidney stones
- 3 ~40% of PH1 patients have already progressed to end-stage renal disease (ESRD) at the time of diagnosis and require a *combined liver-kidney transplant***
- 4 Suppression of *HAO1* is a validated therapeutic approach**
  - *HAO1* gene product acts upstream of *AGXT*
  - Suppression of *HAO1* prevents the formation of oxalate
  - Approach validated by RNAi and natural loss-of-function mutations in *HAO1*
- 5 Strategy: knock-out *HAO1* in liver with a one-time administration of an ARCUS-encoding AAV vector**



# ARCUS Editing Effective in Pre-Clinical Models of PH1

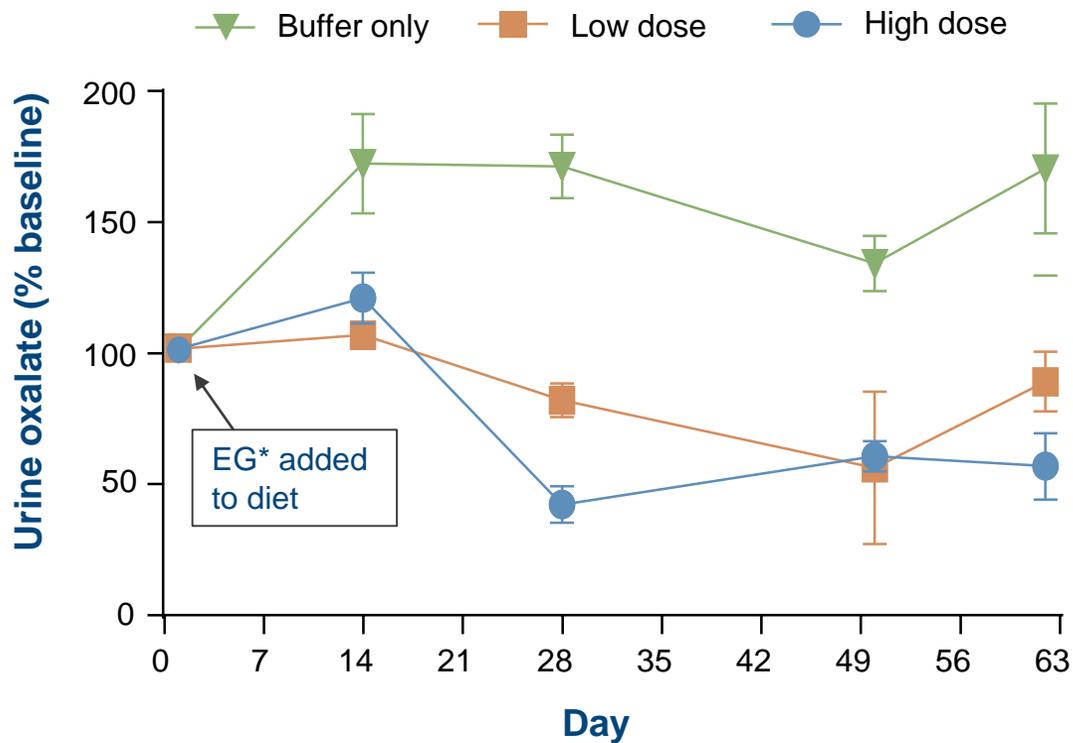


ARCUS treatment resulted in ~70% reduction in urine oxalate in a PH1 mouse model

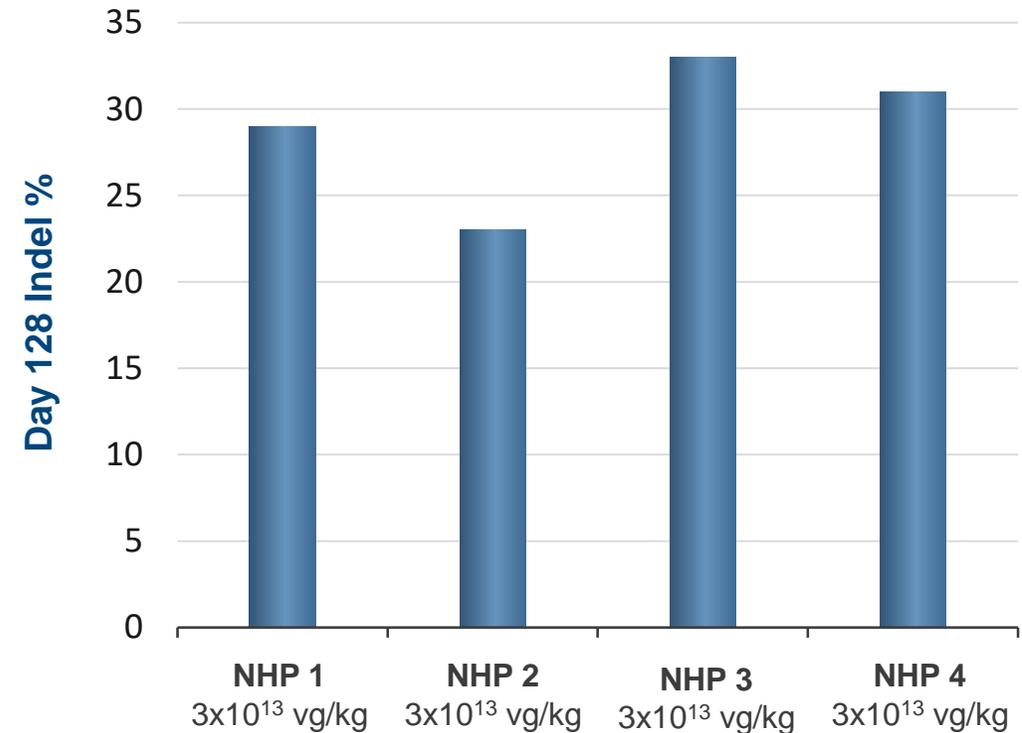
- Prevented formation of renal CaOX crystals

ARCUS efficiently knocked-out the *HAO1* gene in non-human primates following AAV8 delivery

## Mouse model



## Non-human primate



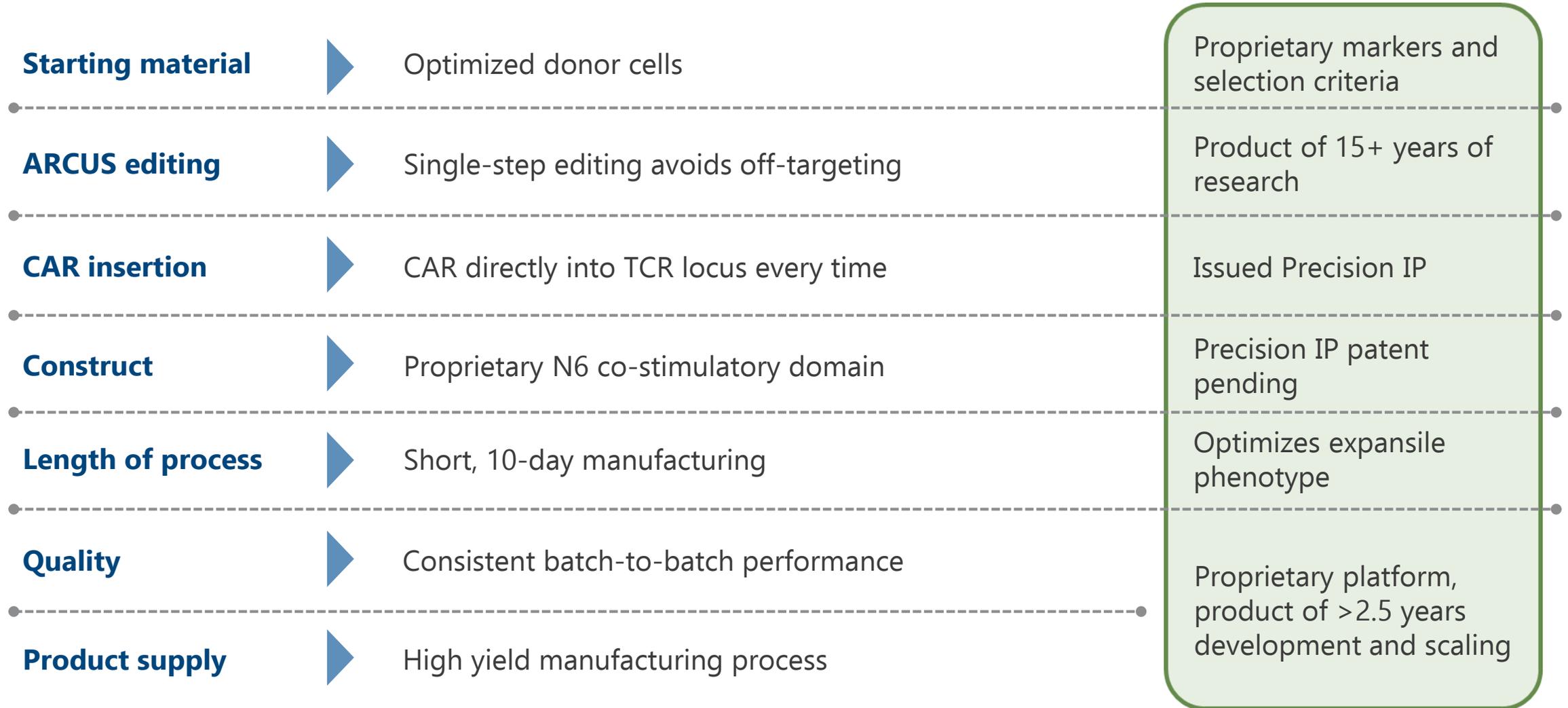


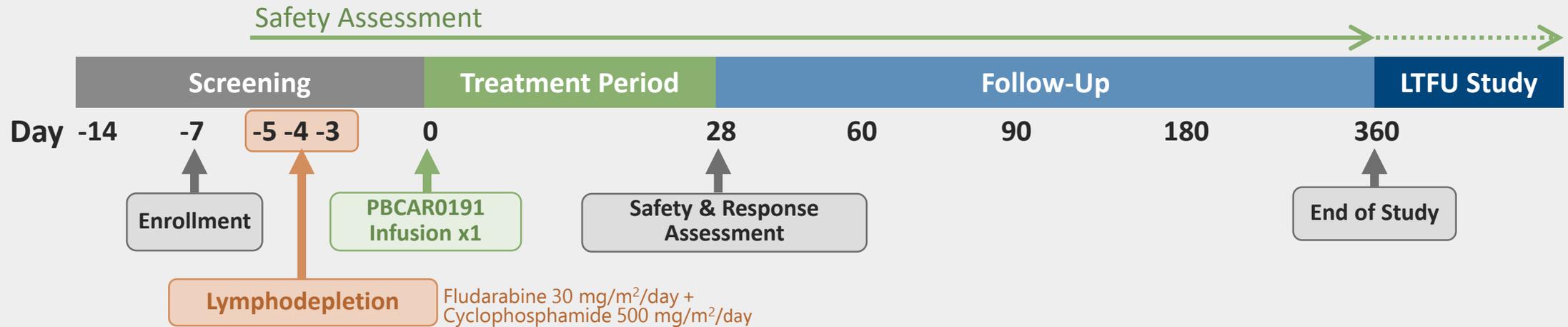
# Overcoming Cancer

## Off-the-Shelf CAR T



# Key Features of Precision's Allogeneic CAR T Platform





## Eligibility

- Adult patients with R/R B-NHL or R/R B-ALL

## Clinical Sites

- Moffitt (Bijal Shah / Mike Jain)
- City of Hope (Anthony Stein / Alex Herrera)
- Dana Farber (Caron Jacobson / Dan DeAngelo)
- MD Anderson (Nitin Jain / Sattva Neelapu)
- +8-10 sites to be initiated

## Objectives

- Primary: safety and tolerability
- Secondary: anti-tumor activity
- Exploratory: expansion, trafficking, and persistence

## Dose Escalation

- **DL1 = 3.0 x 10<sup>5</sup> cells/kg**
- **DL2 = 1.0 x 10<sup>6</sup> cells/kg**
- DL3 = 3.0 x 10<sup>6</sup> cells/kg

# Adverse Events Compare Favorably to Autologous CAR T



<b>System Organ Class</b> <i>Preferred Term, n(%)</i>	<b>NHL</b> (n=6)	<b>B-ALL</b> (n=3)
<b>CRS (Cytokine Release Syndrome) – Grade 1 or Grade 2</b>	2 (33%)	1 (33%)
<b>ICANS (Immune Effector Cell Neurotoxicity) – Grade 1 or Grade 2</b>	0 (0%)	1 (33%)
<b>CRS Grade 3 or higher</b>	0 (0%)	0 (0%)
<b>ICANS Grade 3 or higher</b>	0 (0%)	0 (0%)
<b>GvHD (Graft Versus Host Disease)</b>	0 (0%)	0 (0%)
<b>Infection</b>	0 (0%)	0 (0%)

# PBCAR0191 Summary: NHL Program



Patient ID	Baseline disease burden	Best Response Day ≥28	PFS (Days)*	CRS or ICANS (Mechanistic demonstration of cell expansion)	External PCR Expansion (Study Days +)	Internal Flow Expansion (Study Days +)	Auto CAR T benchmarks
1-NHL-DL1	Aggressive disease (Ki67 65%)	Partial Response	60	None	Positive (Day 3)	Negative <sup>##</sup>	<b>67% ORR</b>
2-NHL-DL1	Bulky aggressive disease (SPD 2,337 / Ki67 90%)	Progressive Disease	N/A	None	Positive (Day 1)	Negative <sup>##</sup>	
3-NHL-DL1	Progressed on Yescarta®	Partial Response	180	CRS Grade 2	Negative <sup>#</sup>	Negative <sup>##</sup>	
4-NHL-DL2	Bulky aggressive disease (SPD 3,693 / Ki67 40%)	Partial Response	60	None	Positive (Day 1-21)	Positive (Day 1-60)	
5-NHL-DL2	Aggressive disease (Ki67 85%)	Progressive Disease	N/A	Hypotension Grade 1; No Fever; ASCTC Gr = Not CRS	Positive (Day 1-10)	Positive (Day 1)	
6-NHL-DL2	Aggressive disease (Ki67 100%)	Complete Response	28+	CRS Grade 1	<LLQ; Detectable (Day 7)	Positive (Days 1-3)	

**Yescarta – NHL (mostly DLBCL, Zuma-1)<sup>1</sup>**  
 54% CR rate / 82% ORR  
 Grade 3/4 CRS 13%;  
 Gr 3/4 Neurotox 28%

**Yescarta – MCL (Zuma-2)<sup>2</sup>**  
 57% CR rate / 82% ORR;  
 Grade 3/4 CRS 18%;  
 Gr 3/4 Neurotox 46%

**Kymriah<sup>3</sup>**  
 40% CR rate / 52% ORR  
 Grade 3/4 CRS 22%;  
 Gr 3/4 Neurotox 12%

\* Progression free survival is estimated at the time of study visit

1 Neelapu NEJM 2017 <https://www.nejm.org/doi/full/10.1056/NEJMoa1707447>

2 Wang, et al., ASH Presentation, Zuma-2, December 2019

3 Schuster NEJM 2019 <https://www.nejm.org/doi/full/10.1056/NEJMoa1804980>

# qPCR performed on DNA extracted from isolated PBMC. Note: extremely low PBMC isolation in 6-NHL-DL2, 7-ALL-DL2, 8-ALL-DL2, and 9-ALL-DL2 yielded low DNA quantities, making interpretation of these results difficult. They are shown for completeness

## Lower limit for CAR+ cells was set as 0.03% of lymphocytes. All positive have ≥0.03%, with highest detected at 0.43%

# PBCAR0191 Summary: ALL Program



Patient ID	Baseline disease burden	Best Response Day ≥28	PFS (Days)*	CRS or ICANS (Mechanistic demonstration of cell expansion)	External PCR Expansion (Study Days +)	Internal Flow Expansion (Study Days +)	Auto CAR T benchmarks
7-ALL-DL2	95% marrow blasts Prior CNS disease	Progressive Disease	N/A	None	Negative <sup>#</sup>	Positive (Day 7)	<b>Yescarta – adult ALL<sup>1</sup></b> 68% CR rate Durability – difficult to assess due to transplant >50% pts in first 6 mos Gr 3/4 CRS 22%; Gr 3/4 Neurotox 22%  <b>Kymriah</b> 60% CR rate (adult ALL) <sup>2</sup> Grade 3/4 CRS 70% Grade 3/4 Neurotox 3.33%
8-ALL-DL2	77% marrow blasts	Progressive Disease	N/A	None	Negative <sup>#</sup>	Negative <sup>##</sup>	
9-ALL-DL2	19.8% marrow blasts	Complete Response	28+	CRS Grade 1; ICANS Grade 2	<LLQ; Detectable (Day 1, 3, 10, 14) <sup>#</sup>	Positive (Day 28)	

\* Progression free survival is estimated at the time of study visit

<sup>#</sup> qPCR performed on DNA extracted from isolated PBMC. Note: extremely low PBMC isolation in 6-NHL-DL2, 7-ALL-DL2, 8-ALL-DL2, and 9-ALL-DL2 yielded low DNA quantities, making interpretation of these results difficult. They are shown for completeness

<sup>##</sup> Lower limit for CAR+ cells was set as 0.03% of lymphocytes. All positive have ≥0.03%, with highest detected at 0.43%

<sup>1</sup> Zuma-3 ASCO Presentation Shah et al., June 2019

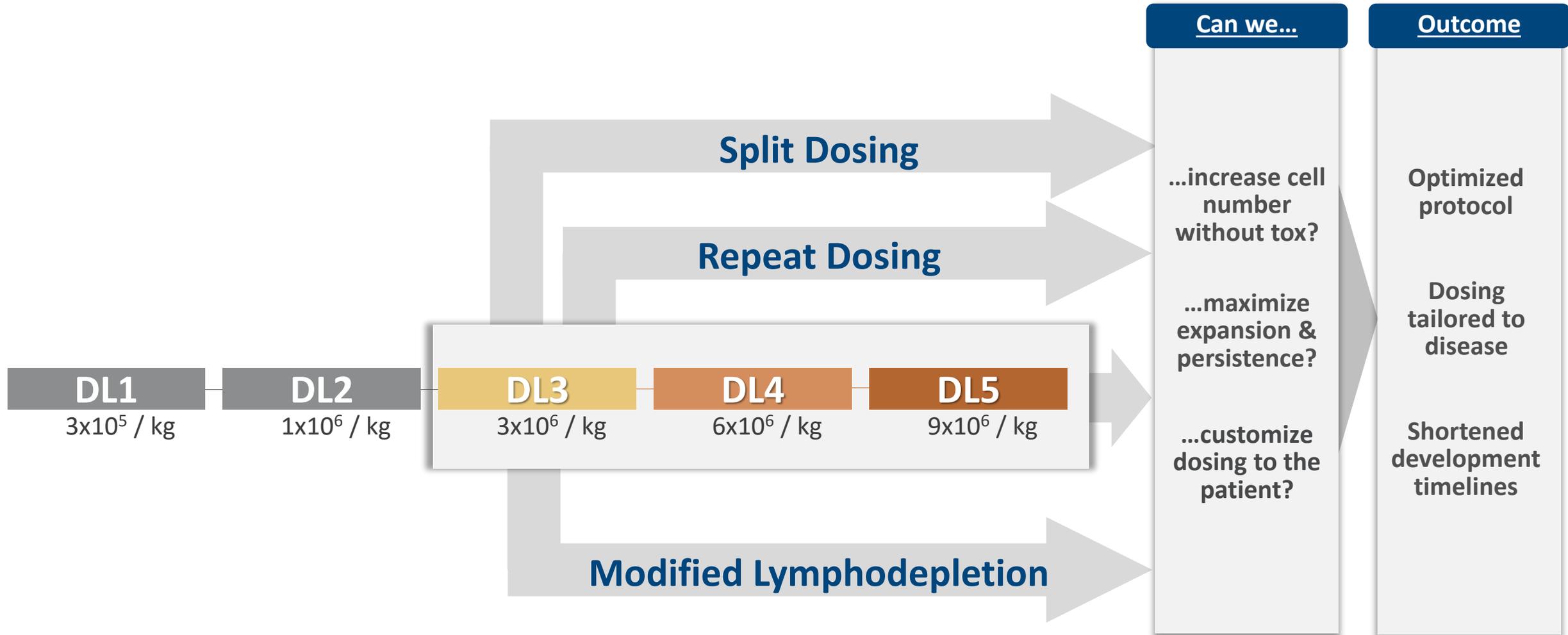
<sup>2</sup> <https://www.clinicaltrials.gov/ct2/show/results/NCT02030847?term=Tisagenlecleucel&cond=Acute+Lymphoid+Leukemia&draw=3&view=results>



## Key Protocol Amendments Recently Proposed to FDA

- 1 Option to **re-dose** after a response and subsequent disease progression
- 2 **Split dosing** into a single lymphodepletion
- 3 **Higher** total doses
- 4 Option to **modify lymphodepletion** (up or down)

# Early Activity and Safety Open Up Multiple Avenues

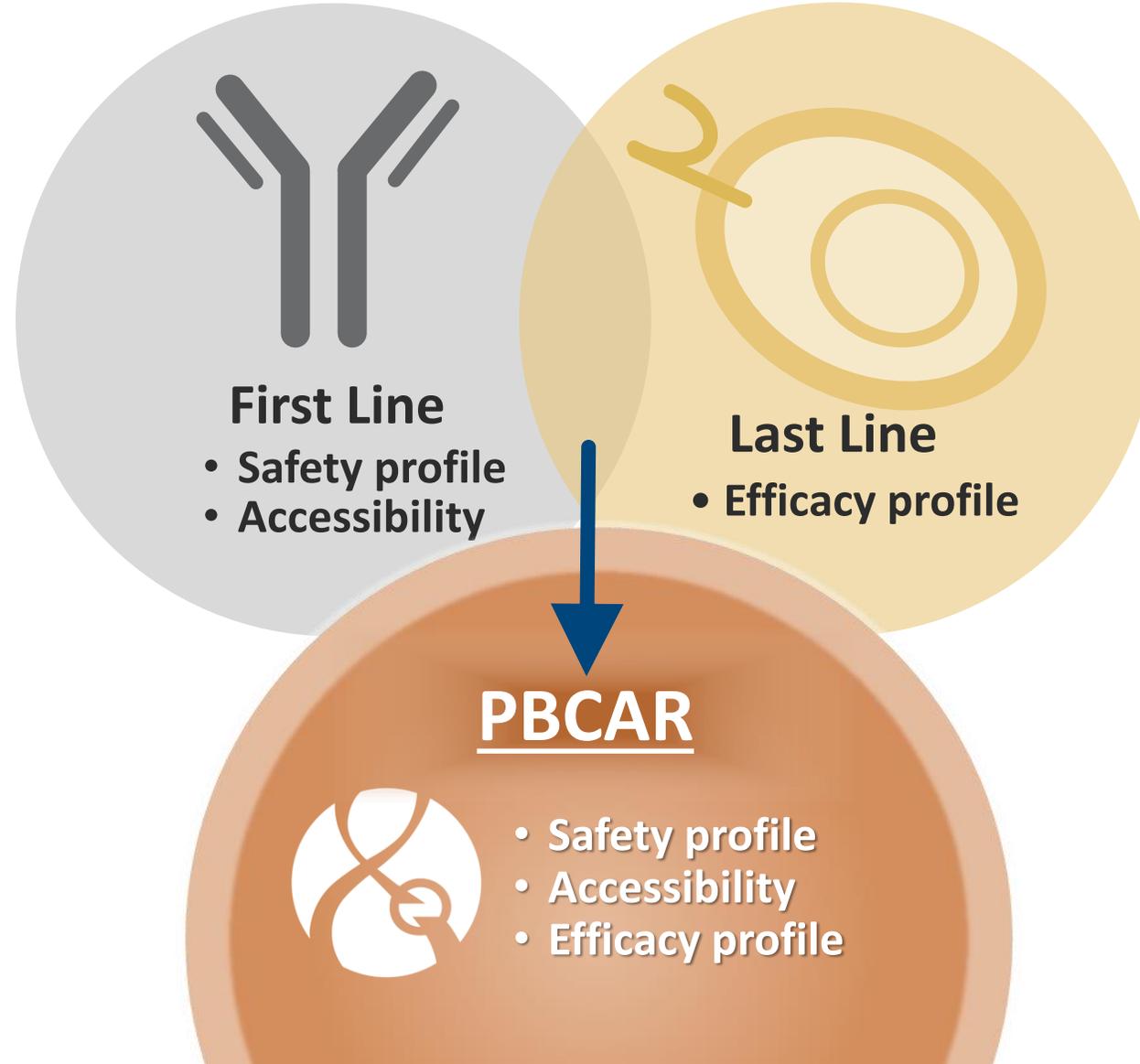


# Off-the-Shelf CAR T: The Best of Both Worlds



Off-The-Shelf Biologics

CAR T



# Off-the-Shelf CAR T Immunotherapy Pipeline



Product Candidates	Program Area	Discovery	Pre-clinical	Clinical	Rights
PBCAR0191 (CD19)	NHL and ALL – Phase 1 Data 2020				 
PBCAR20A (CD20)	NHL, CLL, SLL – Phase 1 Dosing Q1 2020				
PBCAR269A (BCMA)	MM - IND cleared – Phase 1 Dosing 2020				

# Clear Emerging Leadership in Allogeneic CAR T



Objective tumor responses observed at first two dose levels



Interim data suggest safety profile that compares favorably to auto CAR T



Indication of dose-dependent mechanism of action



Evidence allogeneic CAR T can be effective in true “off-the-shelf” setting



Demonstrated ability to manufacture across multiple CAR T targets at scale



Data supports strategy underpinning **entirety of proprietary platform**



# Feed the Planet

Elo Life Systems



## A Human Health Opportunity

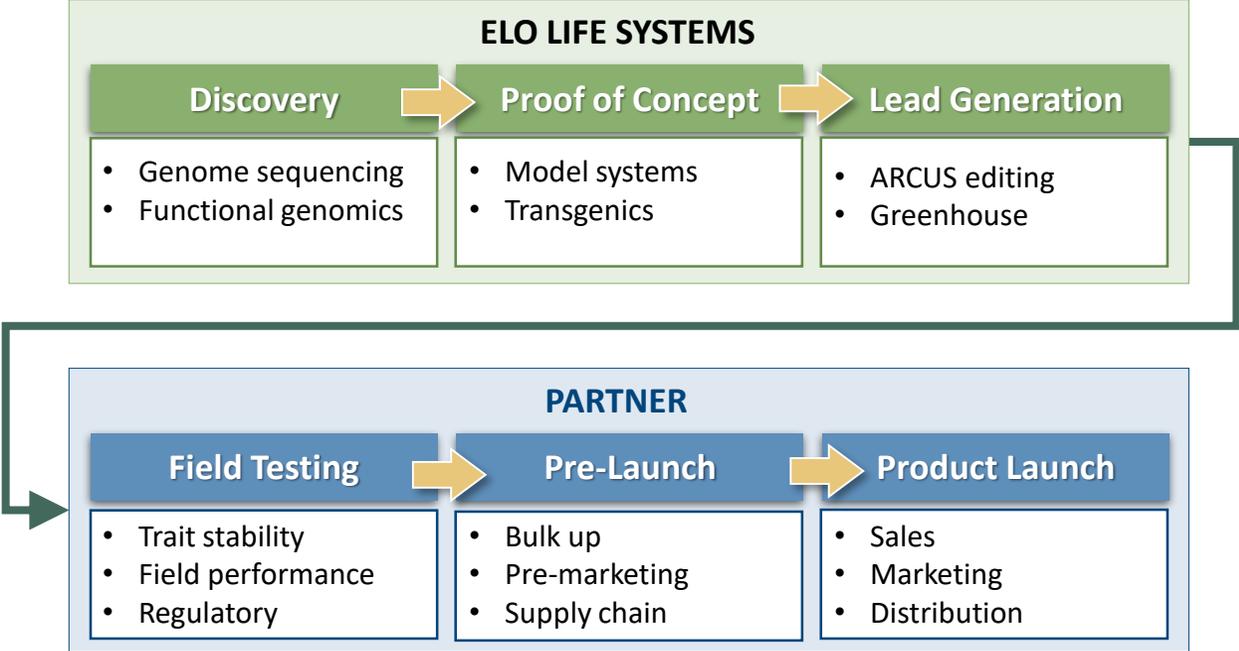
### Food companies need new inputs to respond to:

- **Climate change.** Critical raw materials like citrus and banana are under existential threat
- **Consumer preference.** Consumers are demanding healthier diets

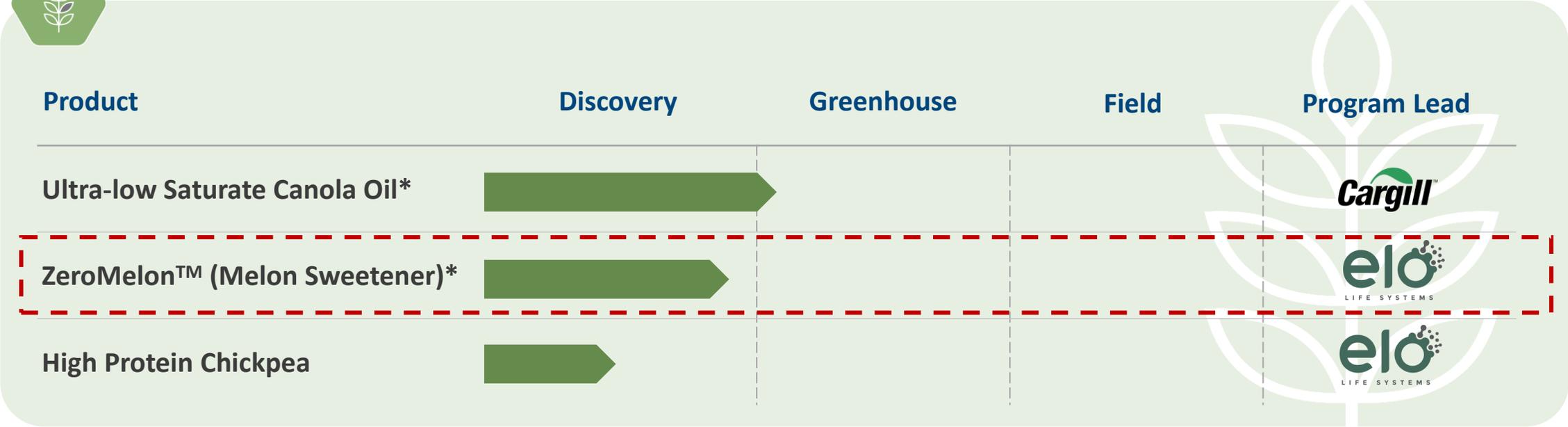
### Elo integrates ARCUS with enabling technologies to create greatly needed improvements to sources of food

- **Partner driven.** Elo partners with end-users early in the life of each new program to ensure market uptake
- **Minimal capital investment.** Projects are primarily partner-funded. Elo has its own facilities and an independent management team

## An Efficient Business Model



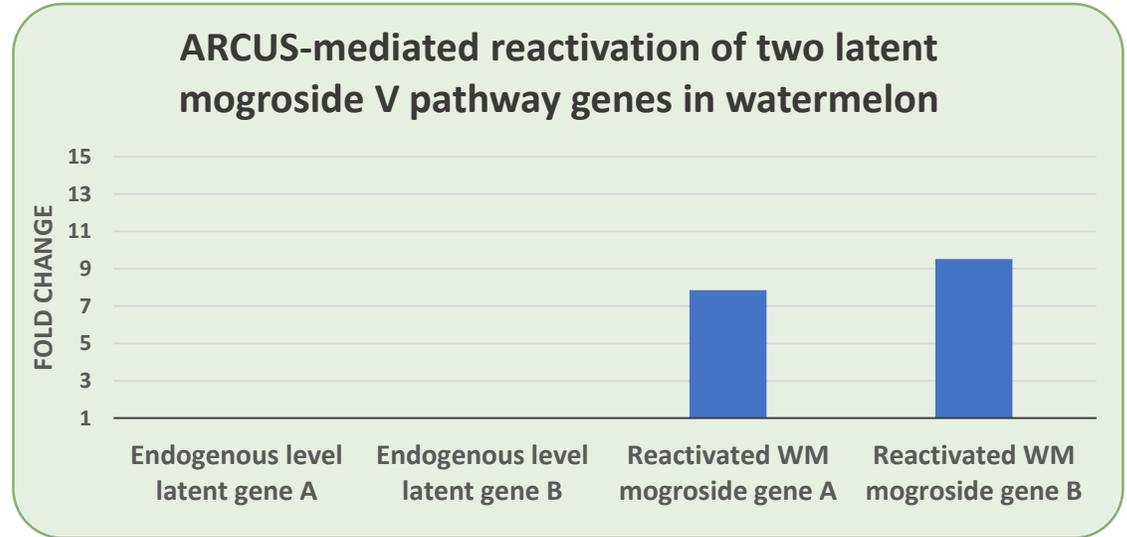
# Food Pipeline



\*Partner funded in whole or in part

# ZeroMelon™: A Zero Calorie Sugar Substitute

- 🌿 Elo's **ZeroMelon™** program leverages ARCUS to reactivate dormant genes in watermelon to produce natural, zero calorie sweetener **mogroside V**
- 🌿 **250x sweeter** than artificial sweeteners / sugar, tasting closer to cane sugar than alternatives
- 🌿 **Substantial opportunity** – global food sweetener market estimated at ~\$82bn by 2024\*
- 🌿 Ability to reactivate mogroside pathway with ARCUS already demonstrated – **validates approach**



## ZeroMelon™ program highlights

- ✓ **Non-GM watermelon** that produces mogroside V at scale
- ✓ Completed **ARCUS-mediated gene reactivation** in publicly-available elite watermelon varieties
- ✓ ZeroMelon™ product development **in progress**

## Potentially rapid path to market

### Major anticipated milestones:

- ✓ **2019** – activation of 2 mogroside V genes in watermelon using ARCUS
- 2021** – greenhouse trials
- 2022** – small scale field trial
- 2023** – large scale, multi-site field trials

***Commercial product possible in 4-5 years***

# Upcoming Milestones Expected Across Portfolio



**IND accepted for BCMA CAR T**

**Initiate dosing for CD20 CAR T**

**Initiate dosing for BCMA CAR T**

**Primary hyperoxaluria candidate selection**

**Start ZeroMelon™ greenhouse studies**

**NHL (CD19) clinical data update**

**ALL (CD19) clinical data update**



**PRECISION**  
BIOSCIENCES

# Key Takeaways



Highly experienced team includes the pioneers in editing



Proprietary ARCUS editing platform with leading specificity and freedom to operate



Independent cGMP manufacturing capabilities



Initial allogeneic CAR T clinical data validates core strategy



Gene correction programs expand upside potential beyond oncology



Q3 cash \$206m, runway into 2H 2021; validating partnerships in each business area



PRECISION  
BIOSCIENCES



Overcome Cancer.



Cure Genetic Disease.



Feed the Planet.



Dedicated To Improving Life