

#### **Forward Looking Statements**

This presentation contains forward-looking statements and information within the meaning of The Private Securities Litigation Reform Act of 1995. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "target," "should," "would," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Forward-looking statements in this presentation include statements regarding the initiation, timing, progress and results of the Company's preclinical and clinical studies and its research and development progress, including initiating the adolescent cohort in the RUBY trial in 2024 and establishing in vivo proof-of-concept for an undisclosed indication in 2024, the timing for the Company's receipt and presentation of data from its clinical trials and preclinical studies, including RUBY clinical updates in mid-2024 and by year-end 2024, the potential of, and expectations for, the Company's product candidates, the timing or likelihood of regulatory filings and approvals, the Company's expectations regarding commercial readiness, and the Company's expectations regarding cash runway. The Company may not actually achieve the plans, intentions, or expectations disclosed in these forward-looking statements, and you should not place undue reliance on these forwardlooking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in these forward-looking statements as a result of various important factors, including: uncertainties inherent in the initiation and completion of pre-clinical studies and clinical trials, including the RUBY and EdiTHAL trials, and clinical development of the Company's product candidates, including reni-cel; availability and timing of results from pre-clinical studies and clinical trials; whether interim results from a clinical trial will be predictive of the final results of the trial or the results of future trials; expectations for regulatory approvals to conduct trials or to market products and availability of funding sufficient for the Company's foreseeable and unforeseeable operating expenses and capital expenditure requirements. These and other risks are described in greater detail under the caption "Risk Factors" included in the Company's most recent Annual Report on Form 10-K, which is on file with the Securities and Exchange Commission, as updated by the Company's subsequent filings with the Securities and Exchange Commission, and in other filings that the Company may make with the Securities and Exchange Commission in the future. Any forward-looking statements contained in this presentation represent Company's views only as of the date hereof and should not be relied upon as representing its views as of any subsequent date. Except as required by law, the Company explicitly disclaims any obligation to update any forward-looking statements.



## Editas Medicine is a Leader in the CRISPR-based Gene Editing Medicine Field



- Reni-cel is an investigational gene editing medicine that is a potential "best in class" treatment for sickle cell disease and beta thalassemia
- Ongoing RUBY and EdiTHAL clinical trials



- Proprietary AsCas12a is a high fidelity and high efficiency CRISPR nuclease
- Core expertise in guide RNA design and chemistry for high precision editing
- Longer-term focus on creating important medicines based on *in vivo* gene editing
- Scaled CMC



- Editas holds exclusive foundational IP for Cas9 and Cas12a for the prevention or treatment of human disease
- Source of non-dilutive capital
  - Recently granted sublicenses to Vertex Pharmaceuticals and Vor Bio



 Leadership team with a proven track record of drug development and commercialization



#### **Strategic Framework**

(From the 2023 J.P. Morgan Healthcare Conference)



Drive reni-cel (EDIT-301) toward BLA and Commercialization

Strengthen and Focus
Discovery to Build *in vivo*Editing Pipeline

Increase Business
Development Activities
and Monetize IP

Long-Term Vision: A Leader in *In Vivo* Programmable Gene Editing



#### **Strategic Transformation Toward Long-Term Vision**

(From the 2023 J.P. Morgan Healthcare Conference)



# Commercial Stage



# Drive reni-cel (EDIT-301) toward BLA and Commercialization

- Continue ex vivo development of reni-cel (EDIT- 301) for SCD, TDT
  - Enroll 20 patients in RUBY study by year-end
  - Provide RUBY and EdiTHAL data updates by mid-year and year-end
- Divest wholly-owned cell therapy program, continue supporting partnered cell therapy programs
- Terminate AAV IRD programs

# Strengthen and Focus Discovery to Build *in vivo*Editing Pipeline

- Focus on in vivo pipeline build
- Hire new CSO with specific expertise aligned with Editas' vision
- Reset discovery and technology group
- Initiate discovery of *in vivo* editing of HSCs and in other tissues

# Increase Business Development Activities and Monetize IP

- Create value through business development to complement core gene editing technology capabilities
- Leverage robust IP portfolio
  - **⊘** Vertex sublicense for exa-cel



#### **2024 Strategic Objectives**

### **Drive reni-cel (EDIT-301) toward BLA and Commercialization**

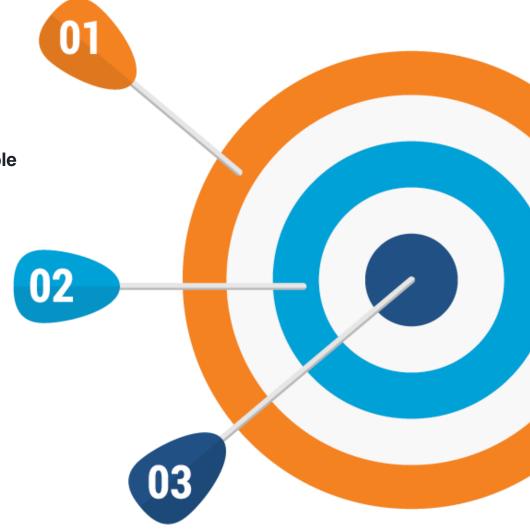
- Continue enrollment and dosing in the RUBY and EdiTHAL trials of reni-cel
- Initiate the adolescent cohort in the RUBY trial
- Present a substantive clinical data set of Sickle cell patients with considerable clinical follow-up in the RUBY study in mid-2024 and by year-end 2024

## Strengthen and Focus Discovery to Build *in vivo* Editing Pipeline

- Establish in vivo preclinical proof-of-concept for an undisclosed indication
  - Focus on disease targets with high probability of technical, clinical, regulatory, and commercial success
  - Initial focus on hematopoietic stem cells (HSCs)

### **Increase Business Development Activities and Monetize IP**

 Derive revenue from the Company's foundational IP, building on the recently announced license agreements with Vertex Pharmaceuticals and Vor Bio





# Sickle Cell Disease (SCD) is an Inherited Life-Threatening Hematological Disorder Manifesting Shortly After Birth



SICKLE CELL DISEASE is a genetic blood disorder caused by mutations in the HBB gene that causes sickling of RBCs; this leads to anemia, hemolysis, and VOEs<sup>1,2</sup>



**UPREGULATION OF FETAL HEMOGLOBIN (HbF)** is a naturally validated therapeutic strategy to control complications of SCD



#### EDITAS EDITS THE HBG1 AND HBG2 PROMOTERS USING AsCAS12a ENZYME, AN APPROACH THAT IS DESIGNED TO:

- Upregulate HbF robustly
- Correct anemia with superior red blood cell production and health vs. BCL11A approach
- Reduce risk of off-target editing with high fidelity and high efficiency proprietary AsCas12a enzyme

Reni-cel is potentially a "best in class" medicine with consistent correction of anemia



#### All Treated RUBY Patients Successfully Engrafted, Showed a Favorable Ruby **Safety Profile**



DEMOGRAPHICS —	(N=11)
Genotype, n(%)	
β <sup>S</sup> /β <sup>S</sup>	11 (100)
Sex, n (%)	
Female	6 (54.5)
Age, years, mean (SD)	27.6 (4.2)
Severe VOEs, pre-study annual rate*, mean (SD)	3.9 (1.4)
INFUSION AND ENGRAFTMENT —	(N=11 <sup>†</sup> )
Total reni-cel dose administered, ×10 <sup>6</sup> CD34 <sup>+</sup> cells/kg, mean (SD)	5.2 (2.5)
Follow-up duration, months, mean (SD)	6.5 (5.3)
Time to neutrophil engraftment <sup>†, ‡</sup> , days, mean (SD)	23.7 (2.8)
Time to platelet engraftment <sup>†,§</sup> , days, mean (SD)	26.1 (7.7)

- Safety profile is consistent with myeloablative busulfan conditioning and autologous HSCT
- No serious adverse events (SAEs) related to reni-cel were reported after reni-cel infusion

Data cutoff November 22, 2023.

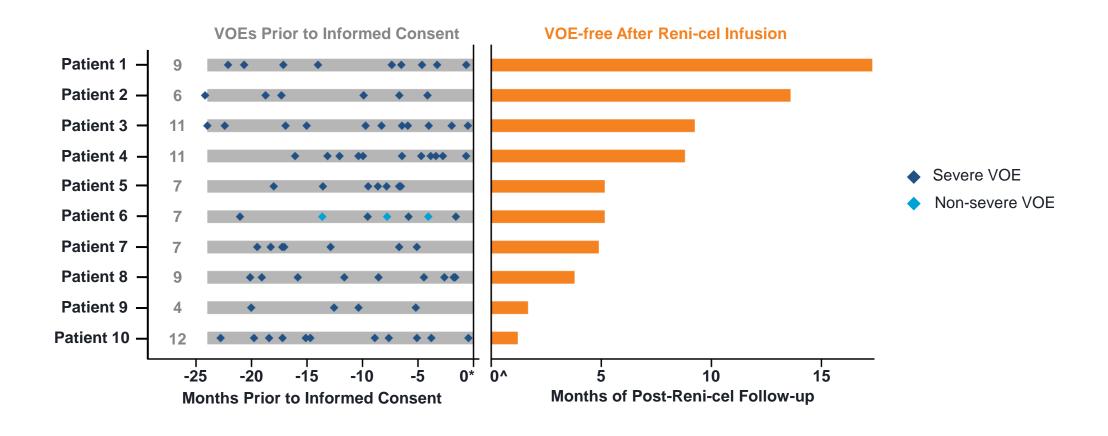
HSCT, hematopoietic stem cell transplant; reni-cel, renizgamglogene autogedtemcel; SCD, sickle cell disease; SD, standard deviation; SAE, serious adverse event; VOE, vaso-occlusive event. Hanna R et al. Poster presented at ASH 2023: San Diego, CA, USA, 9-12 December,



<sup>\*</sup>The pre-study period is defined as the 2-year period prior to informed consent. †One patient had 23 days of follow-up after infusion as of the data cut; neutrophil engraftment and platelet engraftment were not achieved yet; engraftment values are therefore based on n=10. ‡Three consecutive measurements with absolute neutrophil count (ANC) ≥0.5 × 10<sup>9</sup>/L. ⁵Three consecutive measurements with platelet count ≥50 × 10<sup>9</sup>/L starting at least 7 days after the platelet transfusion, and 10 days after thrombopoietin (TPO). No TPO was used for patients after reni-cel infusion.

#### All Treated RUBY Patients are VOE-free Since Reni-cel Infusion



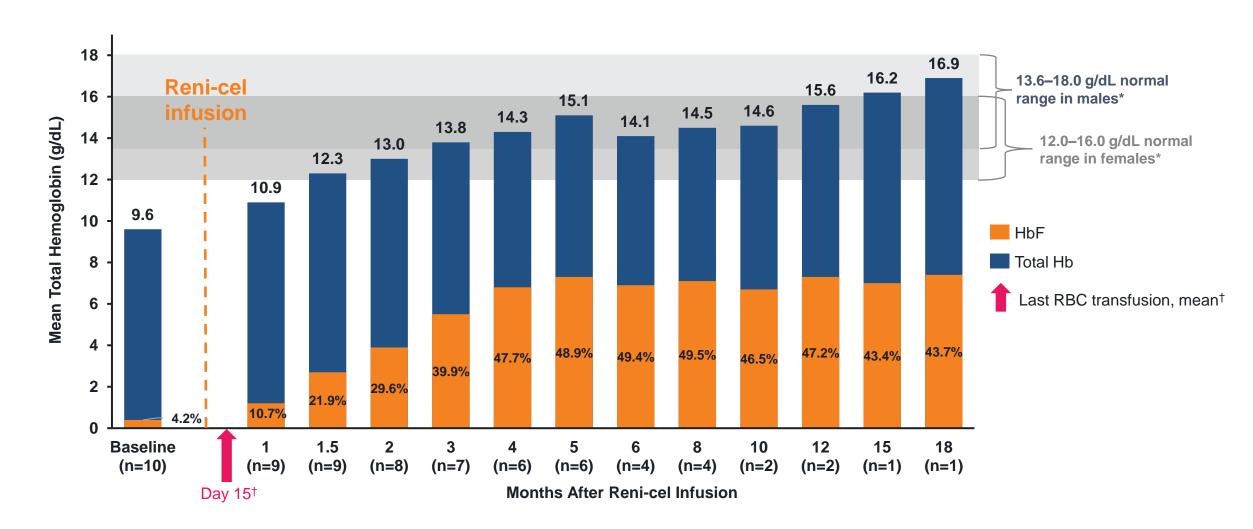


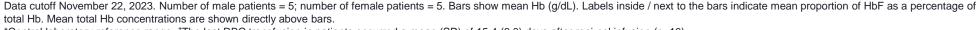
All 10 patients who reached the Month 1 visit have been VOE-free since reni-cel infusion

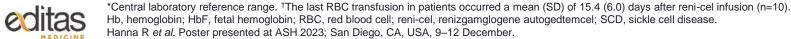


## RUBY Patients Show Total Hb Rapidly Returning to the Normal Range and Clinically Meaningful Improvements in HbF Levels of >40%









## EdiTHAL Patients Successfully Engrafted, Experienced Similar Engraftment and Similar Safety Profile to RUBY Patients



- DEMOGRAPHICS	(N=6)
Genotype, n(%)	
βο/βο	2 (33.3)
Non- $\beta^0/\beta^{0^*}$	4 (66.7)
Sex, n (%)	
Female	4 (66.7)
Age, years, mean (SD)	18.8 (0.9)
RBC transfusion volume, pre-study annual rate <sup>†</sup> , mL/kg/year, mean (SD)	162.3 (51.9)

 Safety profile is consistent with myeloablative busulfan conditioning and autologous HSCT

 No serious adverse events (SAEs) related to reni-cel were reported after reni-cel infusion

INFUSION AND ENGRAFTMENT	(N=6 <sup>‡</sup> )
Total reni-cel dose administered, ×10 <sup>6</sup> CD34 <sup>+</sup> cells/kg, mean (SD)	7.7 (2.2)
Follow-up duration, months, mean (SD)	4.1 (2.5)
Time to neutrophil engraftment <sup>§</sup> , days, mean (SD)	25.5 (3.6)
Time to platelet engraftment <sup>‡,∥</sup> , days, mean (SD)	36.6 (11.8)

Data cutoff November 28, 2023.

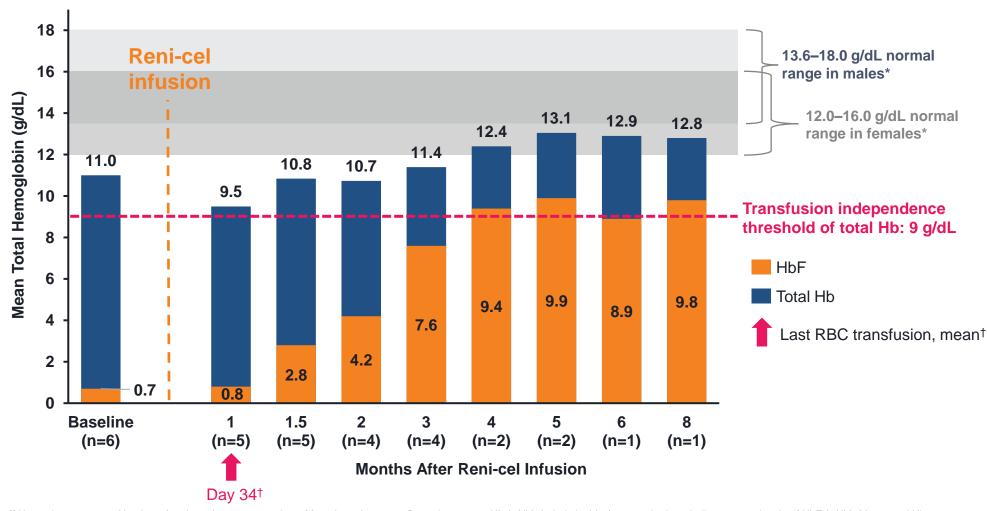
\*Non-β<sup>0</sup>/β<sup>0</sup> includes β<sup>0</sup>/β<sup>+</sup> (n=3) and β<sup>E</sup>/β<sup>0</sup> (n=1). †The pre-study period is defined as the 2-year period prior to informed consent. ‡One patient had 36 days of follow-up after infusion as of the data cut; neutrophil was engrafted, but platelet engraftment was not achieved yet; platelet engraftment values are therefore based on n=5. §Three consecutive measurements with absolute neutrophil count (ANC) ≥0.5 × 10<sup>9</sup>/L. ¶Three consecutive measurements with platelet count ≥20 × 10<sup>9</sup>/L starting at least 7 days after the platelet transfusion, and 10 days after thrombopoietin (TPO). No TPO was used for patients after reni-cel infusion. RBC, red blood cell; reni-cel, renizgamglogene autogedtemcel; SD, standard deviation; TDT, transfusion-dependent β-thalassemia; SAE, serious adverse event.

Hanna R *et al.* Poster presented at ASH 2023; San Diego, CA, USA, 9–12 December.



## **EdiTHAL** Patients Had Early and Robust Increase of Total Hb Above the Transfusion Independence Threshold





Data cutoff November 28, 2023. Number of male patients = 2; number of female patients = 4. Bars show mean Hb (g/dL). Labels inside / next to the bars indicate mean levels of HbF (g/dL). Mean total Hb concentrations are shown directly above bars.



## Reni-cel's Rational Design: Target Selection AND CRISPR Enzyme Matter in Building a Medicine to Give Best Outcomes to Patients





TARGET

HBG1 and HBG2

RBC Production

Normal

Reduced

Normal

Reduced

Normal

Reduced

Normal

Reduced

Normal

Reduced

Normal

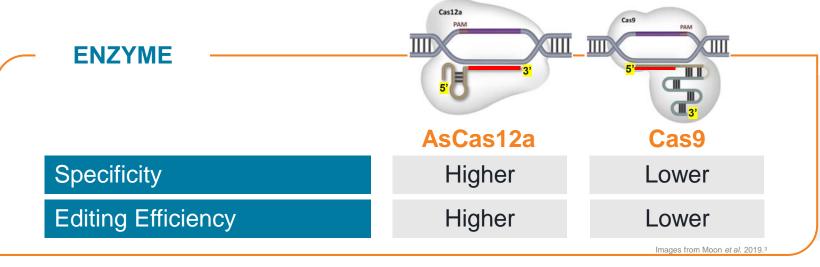
Normal

Normal

Normal

Normal

HBG1 and HBG2 promoters are a more appropriate genomic target versus BCL11A for RBC production<sup>1,2</sup>



AsCas12a is a differentiated CRISPR nuclease with higher specificity and efficiency compared with Cas9<sup>1,4</sup>



#### **Key Takeaways**











Reni-cel drives early, robust correction of anemia to normal physiological range of total Hb for SCD

Reni-cel drives robust sustained increases in HbF >40%

No VOEs seen to date in all dosed SCD patients





Reni-cel safety profile consistent with myeloablative busulfan conditioning and autologous HSCT

Initial Hb and HbF responses are consistent in SCD and TDT patients at the same follow-up time points



#### **2024 Strategic Objectives**

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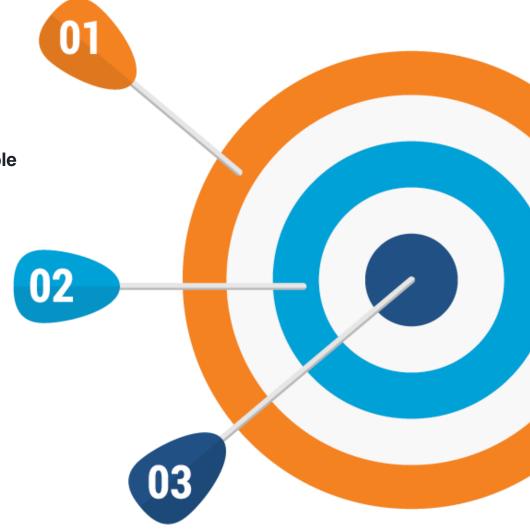
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#### **Investment Highlights**

World leading gene editing platform supported by foundational IP estate.

Shareholder value driven team with a proven track record of drug development & commercialization, strong domain expertise and focus on execution.

Lead asset renizgamglogene autogedtemcel (reni-cel) a potentially differentiated treatment for sickle cell disease and beta thalassemia.

Recent data at the American Society of Hematology (ASH) Annual Meeting supporting differentiation with two additional clinical data updates expected in 2024.

Disciplined, longer-term focus on creating important medicines for people living with serious diseases based on *in vivo* gene editing.

Strong cash position with operational runway into 2026.

**Groundbreaking Science** 

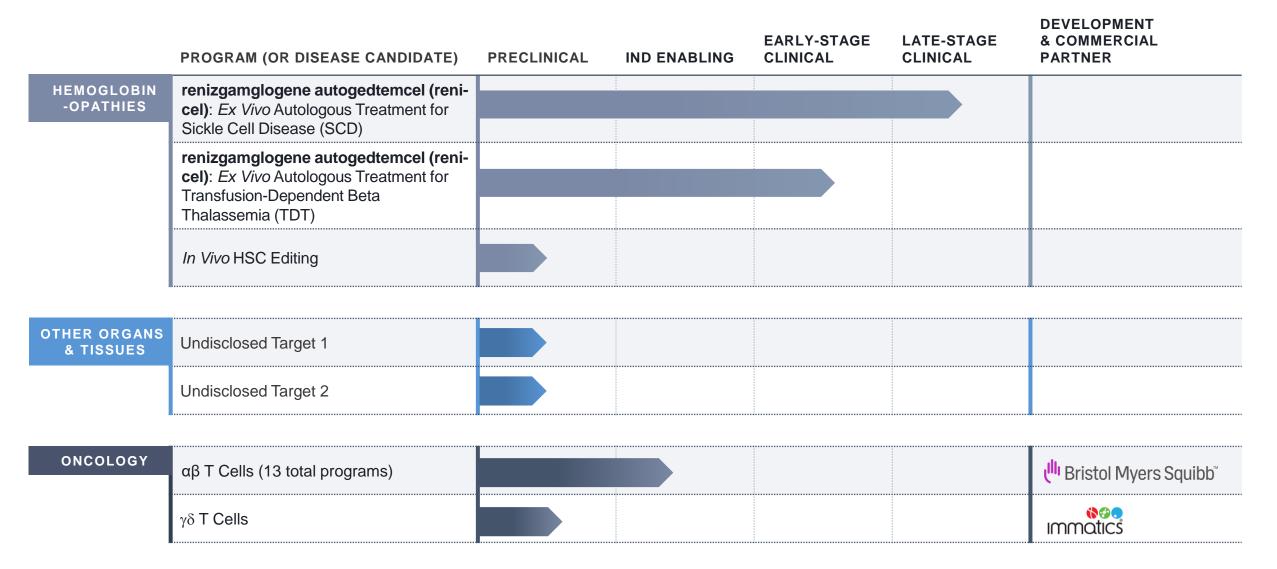
**Strong Leadership Team** 

**Commercial Focus** 





#### **Programs Positioned for Clinical Success**





#### **Experienced Team Focused on Delivering Shareholder Value**



Gilmore O'Neill, M.B, M.M.Sc.
Chief Executive Officer
Prior experience: Sarepta • Biogen



Linea Aspesi
Chief People Officer
Prior experience: Forma · Saniona · Sobi ·
Sanofi/Genzyme



Linda Burkly, Ph.D. Chief Scientific Officer Prior experience: Biogen



Caren Deardorf
Chief Commercial and Strategy Officer
Prior experience: Magenta • Ohana • Biogen



Chi Li, Ph.D., MBA, RAC
Chief Regulatory Officer
Prior experience: Celularity • Allergan • Bayer



Erick Lucera
Chief Financial Officer
Prior experience: AVEO Oncology •
Valeritas • Aratana



Baisong Mei, M.D., Ph.D.
Chief Medical Officer
Prior experience: Sanofi • Biogen • Bayer



Charlene Stern, Ph.D., J.D.
General Counsel
Prior experience: AVEO Oncology • Goodwin •
Foley Hoag

