

Stephanie and Tristan LIVING WITH SICKLE CELL DISEASE

# **Strategic Update**











### **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 Company's intent to partner or out-license reni-cel and any benefits resulting therefrom, the initiation, timing, progress and results of the Company's preclinical and clinical studies and its research and development programs, the potential of, and expectations for, the Company's product candidates, including any in vivo gene edited medicines the Company may develop, and the Company's expectations regarding cash runway. The Company may not actually achieve the plans, intentions, or expectations disclosed in these forwardlooking statements, and you should not place undue reliance on these forward-looking 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 preclinical studies and clinical trials, and clinical development of the Company's product candidates; whether interim results from preclinical studies will be predictive of the final results of the study or the results of any future clinical trials; 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.



### **Agenda and Speakers**

Welcome and Strategic Focus Update

In vivo vision, strategy, and preclinical data

**Business Development and Financing** 

**Closing Remarks** 

Q&A

### **SPEAKERS**



Gilmore O'Neill, MB, MMSc President and Chief Executive Officer



Linda Burkly, P.h.D Chief Scientific Officer



Erick Lucera, MBA, MS
Chief Financial Officer



## **2024 Strategic Objectives**

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

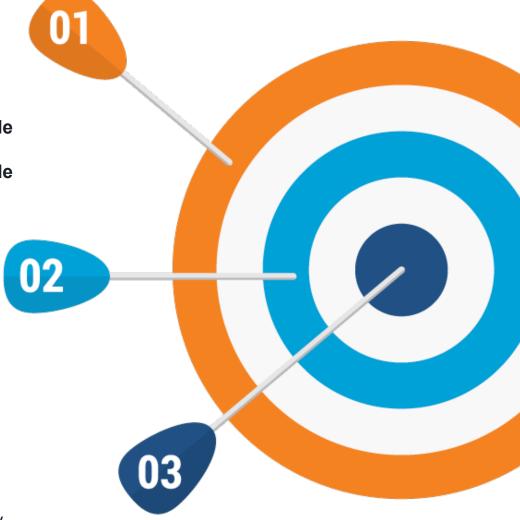
- ✓ Continue enrollment and dosing in the RUBY and EdiTHAL trials of reni-cel
- ✓ Initiate the adolescent cohort in the RUBY trial
- ✓ Present a substantive clinical data set of Sickle cell patients with considerable clinical follow-up in the RUBY study in mid-2024
- Present a substantive clinical data set of Sickle cell patients with considerable clinical follow-up in the RUBY study 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 previously announced license agreements with Vertex Pharmaceuticals and Vor Bio





# Leverage Cutting Edge Technology to Become a Leader in *In Vivo* Programmable Gene Editing



Broadens target profile to treat a larger, global patient population than *ex vivo* medicines with potentially **best-in-class**, **first-in-class** medicines



Reduces treatment burden for patients and healthcare system removing need for conditioning with chemotherapy, infertility concerns, and isolation during treatment

Editas' clinical validation with reni-cel provides a strong foundation for developing a first and best-in-class *in vivo* medicine



## Editas' In Vivo Gene Editing Mission

### **OUR IN VIVO STRATEGY:**

Deliver first-to-market and best-in-class in vivo gene edited medicines as cures for genetically determined diseases



Functional Gene Upregulation in Targeted Tissues / Cells



Develop First-in-Class and Bestin-Class Medicines

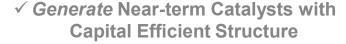


Leverage Established Gene Editing Capabilities

✓ Deliver Differentiated Scientific Value









- Sickle cell disease & beta thalassemia, targeting hematopoietic stem cells (HSCs)
- Genetic loss of function or deleterious mutations in other tissues
- "Programmable" targeted lipid nanoparticle (tLNP) delivery

#### Select targets that

- Meaningfully differentiate from the standard of care and
- Address unmet need in untapped genetic diseases via upregulation strategy

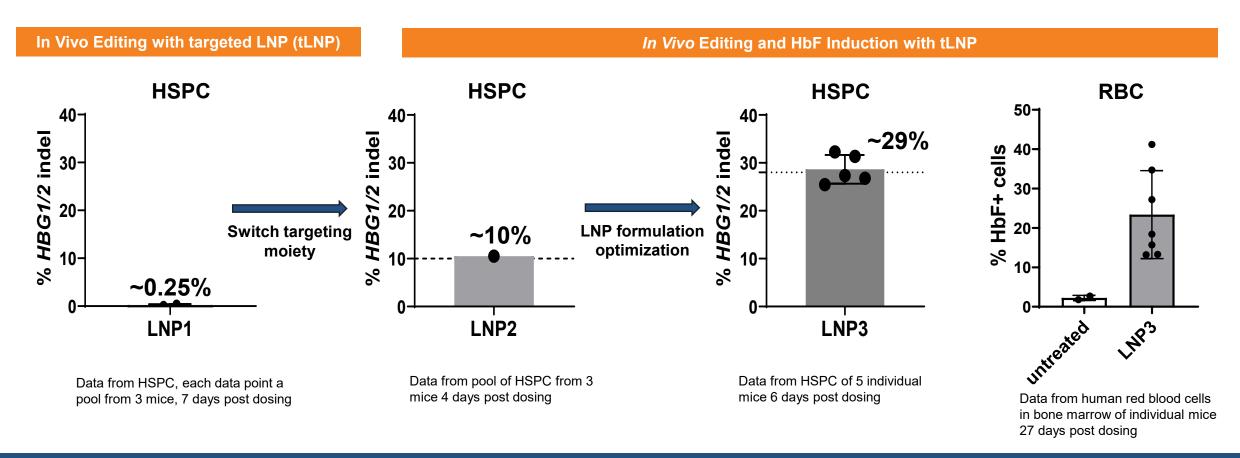
Uniquely positioned to lead in vivo market by leveraging

 Guide RNA, AsCas12a editing enzyme, messenger RNA, and delivery technology

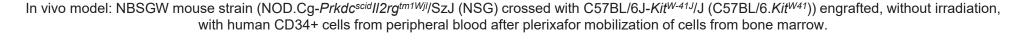
Differentiated through a Capital Efficient Drive to Meaningful Data, Aiming for First- and Best-in-Class Medicines, and Broad Tissue Access



# High Levels of *In Vivo* HSPC Editing and HbF Induction Achieved with a Novel LNP and Targeting Strategy in Mice with Human Hematopoietic Stem Cells



Preclinical PoC achieved as shown by the functional outcome of HbF induction after a single dose of tLNP, editing the clinically validated *HBG1/2* target by AsCas12a enzyme with novel HSC targeting strategy and proprietary LNP





# Preclinical POC Data in *In Vivo* HSC Editing Leverages Editas' Gene Editing Expertise and Provides Foundation for LNP Platform

Potential for Best-in Class, First-in-Class In Vivo Medicine for Sickle Cell Disease



Leveraging reni-cel experience with validated target and enzyme that provides for development of a differentiated medicine for sickle cell disease and beta thalassemia



Demonstrated *in vivo* capabilities with devised novel HSC targeting strategy and proprietary LNP to deliver editing cargo



**Produced competitive** preclinical data set that outperforms data currently in the public domain

### **Proprietary LNP Platform**

- Foundation for a potential LNP Platform for Delivery to Extrahepatic Tissues
- Ability to deliver gene editing cargo with HSC targeting moiety conjugated to our propriety LNP Platform
- Potential to deliver cargo to other tissues and cell types of interest



# **Building for a Capital Efficient Gene Editing Company**

Seeking Alternatives to Fully Owned Reni-cel Launch



#### **Partner and Out-license Reni-cel Globally**

- More effectively drives to commercialization
- Maintains long-term viability of Editas

Financing a Capital
Efficient in vivo Editing
Pipeline



### **Capture Curative Opportunity**

- Broaden patient access
- Efficient development and commercialization cost model

Continuing Focus on Business Development and IP Monetization



#### **Leverage Foundational IP Estate**

- Business development to drive IP licensing
- Continue to seek anti-dilutive financing opportunities





# Recent Success from IP Financing...





Provides upfront cash of \$57M via non-dilutive financing

Editas Medicine Announces \$50+ Million Monetization Financing with DRI Healthcare Trust



Strengthens balance sheet with non-dilutive capital to enable further pipeline development and related strategic priorities

October 03, 2024 18:00 ET | Source: Editas Medicine, Inc.





Potential to unlock future business development and licensing opportunities

...Demonstrates Continued Execution to Leverage Foundational IP Estate to Access Non-Dilutive Capital



# **Closing Remarks**



**Gilmore O'Neill, MB, MMSc**President and Chief Executive Officer
Editas Medicine



## **Acknowledgements**

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### **Questions & Discussion**



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