

### **DISCLAIMER**

Various statements in this release concerning Rocket's future expectations, plans and prospects, including without limitation, Rocket's expectations regarding its guidance for 2022 in light of COVID-19, the safety, effectiveness and timing of product candidates that Rocket may develop, to treat Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD), and Danon Disease, and the safety, effectiveness and timing of related pre-clinical studies and clinical trials and related data readouts, may constitute forward-looking statements for the purposes of the safe harbor provisions under the Private Securities Litigation Reform Act of 1995 and other federal securities laws and are subject to substantial risks, uncertainties and assumptions. You should not place reliance on these forward-looking statements, which often include words such as "believe," "expect," "anticipate," "intend," "plan," "will give," "estimate," "seek," "will," "may," "suggest" or similar terms, variations of such terms or the negative of those terms. Although Rocket believes that the expectations reflected in the forward-looking statements are reasonable, Rocket cannot guarantee such outcomes. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including, without limitation, Rocket's ability to monitor the impact of COVID-19 on its business operations and take steps to ensure the safety of patients, families and employees, the interest from patients and families for participation in each of Rocket's ongoing trials, our expectations regarding when clinical trial sites will resume normal business operations, our expectations regarding the delays and impact of COVID-19 on clinical sites, patient enrollment, trial timelines and data readouts, our expectations regarding our drug supply for our ongoing and anticipated trials, actions of regulatory agencies, which may affect the initiation, timing and progress of pre-clinical studies and clinical trials of its product candidates, Rocket's dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, the outcome of litigation, and unexpected expenditures, as well as those risks more fully discussed in the section entitled "Risk Factors" in Rocket's Annual Report on Form 10-K for the year ended December 31, 2021, filed February 28, 2022 with the SEC and subsequent filings with the SEC including our Quarterly Reports on Form 10-Q. Accordingly, you should not place undue reliance on these forward-looking statements. All such statements speak only as of the date made, and Rocket undertakes no obligation to update or revise publicly any forward-looking statements, whether as a result of new information, future events or otherwise.



## Agenda

Introduction ----- Gaurav Shah, CEO

- Strategic rationale
- Transaction details
- Synergies

About Renovacor ----- Gaurav Shah, CEO

- BAG3 program
- Preclinical data
- Development

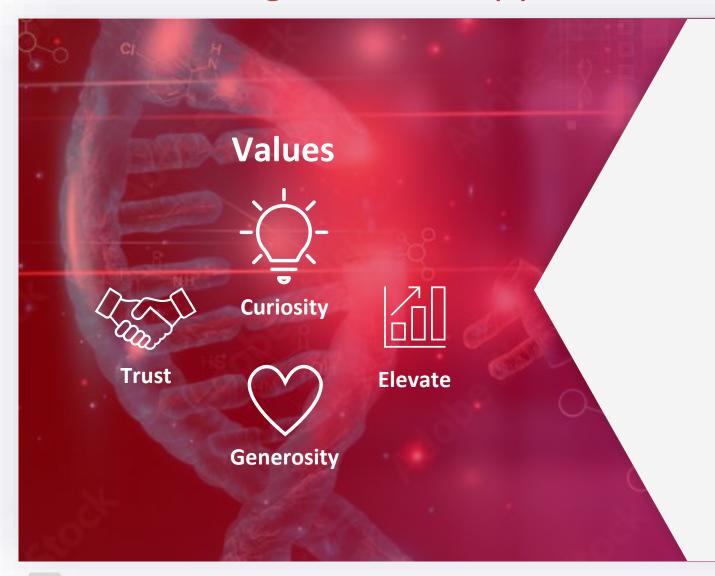
Conclusion ----- Gaurav Shah, CEO

- Next Steps
- Financial position
- Q&A

Martin Wilson, General Counsel & Chief Compliance Officer



## **Vision:** Seeking Gene Therapy Cures



### **Mission**

To develop first-in-class and best-in-class curative gene therapies for patients with devastating diseases



## Rocket Pharma to Acquire Renovacor as a Strategic Move

Adds BAG3 opportunity to create leading cardiac AAV gene therapy franchise by expanding clinical/near clinical assets

#### Strategic Rationale – the 3Ps, Pipeline, Property (Intellectual), People



**P**ipeline

• Significant unmet medical need in BAG3-DCM with ability to be first, best and only-in-class with clear on-target MOA and commercial opportunity similar to Danon Disease



Property (Intellectual)

• Broad IP portfolio provides freedom to operate and optionality for addressing BAG3-associated dilated cardiomyopathy (BAG3-DCM)



**P**eople

Acquisition adds cardiac and gene therapy expertise to Rocket's growing team

Expected to add approximately \$38M in projected net cash at closing, extending cash runway into 2Q'24



### **Transaction Overview**

- Rocket has signed a definitive agreement to acquire Renovacor in an allstock transaction at an exchange ratio of 0.1676, an implied per-share value of \$2.60
  - RCOR shareholders to own 4.6% of pro forma entity
  - Implied total equity value of approximately \$53M at close based on common shares outstanding
- Offer preserves cash resources of pro forma company and allows Renovacor shareholders to share in future upside
- Proposal has been approved by the Boards of Directors of both Rocket and Renovacor, with voting agreements in place with significant shareholders

Transaction expected to close by first quarter of 2023



# Addition of Renovacor's Programs and Capabilities Aligns with Our Core Strategy

Renovacor Acquisition Matches our Mission of Developing First-, Best- and Only-in-Class Therapies for Rare Diseases With Extensive Unmet Needs



### Strong science, carefullyselected assets and smart execution

- Clear MOA: corrected protein made in target cells responsible for disorder caused by single gene mutations
- Well-defined, achievable clinical endpoints
- CMC compatible with Rocket's in-house AAV cGMP manufacturing capabilities



# Personnel with proven development expertise

- Deep cardiac and gene therapy scientific expertise complementary to Rocket's experience
- Strong drug development track record
- Engagement with health authorities to outline a predictable review pathway



# Renovacor Adds High Value Assets and Capabilities to Rocket's Cardiac AAV Franchise

DETERMINANTS OF SUCCESS IN GENE THERAPY	RENOVACOR VALUE ADD
Promising therapeutic candidates targeting genetically-defined diseases	BAG3-DCM – near-clinical program with compelling preclinical data in monogenic indication of significant unmet need
Well-defined developmental and regulatory pathway, with clear endpoints	Monogenic target disease (BAG3-DCM) with relevant functional endpoints clearly impacting patient prognosis
Proprietary technologies imparting competitive advantage	Proprietary BAG3 mouse model accelerates development efforts and strengthens IP moat in disease area
Scientific and developmental expertise in modality and therapeutic area	Additional personnel and capabilities in cardiac AAV gene therapy bolster Rocket's industry-leading cardiac platform



# Acquisition Strengthens Cardiac AAV Gene Therapy Franchise

#### **Criteria used to select programs**



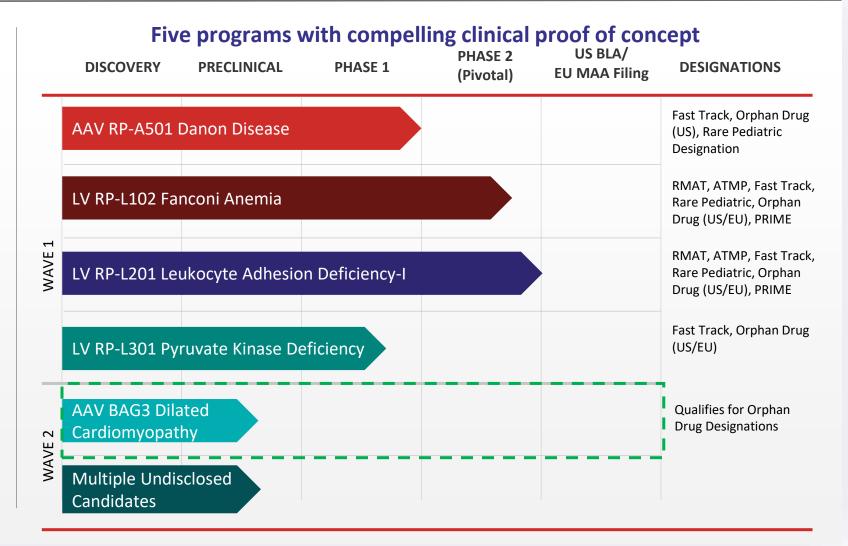
First-, best- and only-in-class



On-target MOA; clear endpoints



Sizeable market to maximize patient impact





## **BAG3** Regulates Critical Functions in Cardiomyocytes

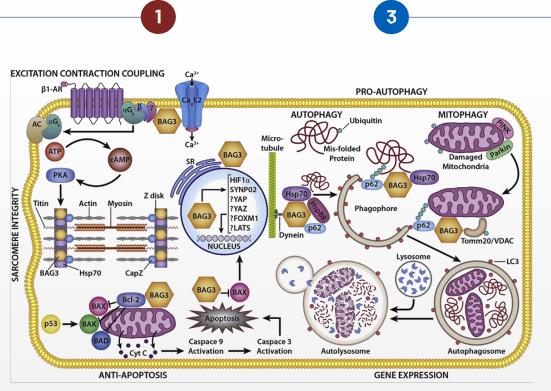
#### **Cardiac contractility**

Enhances contractility by linking the  $\beta$ -adrenergic receptor and L- type  $Ca^{2+}$  channel

2

#### **Structural support**

Provides support for the sarcomere by linking actin myofibrils with the Z-disc



#### **Protein quality control**

Facilitates autophagy as a cochaperone with heat shock proteins, recycling misfolded proteins

4

#### **Anti-apoptosis**

Inhibits apoptosis (programmed cell death) through binding of BCL2

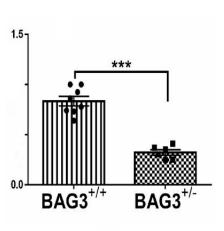
We believe that a gene therapy approach is best positioned to restore the broad biological functions of BAG3 in the heart



# AAV9 BAG3 Prevents the Onset of Cardiac Impairment in Genetic Mouse Model of BAG3-Associated DCM

# BAG3 +/- mice have ~50% of BAG3 protein and develop a reduced ejection fraction (EF)

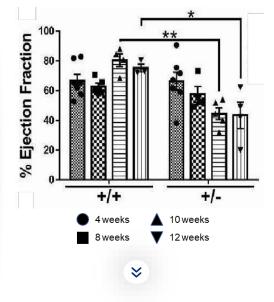
#### **BAG3** protein levels



~50% BAG3 protein levels seen in

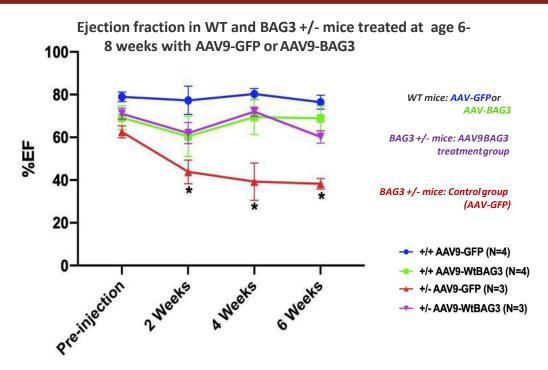
BAG3 +/- mice

#### **Ejection fraction**



BAG3 +/- mice develop reduced EF, recapitulating the DCM clinical phenotype

# AAV9 BAG3 has demonstrated the prevention of onset of reduced ejection fraction



\*p=.04, .01 and .003 respectively at 2, 4 and 6 weeks for +/- AAV9-GFP vs. +/- AAV9-BAG3 arms; dose =  $1 \times 10^{13}$  genome copies (ac). Weeks on X-axis denote time since treatment.



### BAG3-DCM Represents a Significant Market with Unmet Need

- Dilated cardiomyopathy (DCM) is the most common form of cardiomyopathy
- 20-50% of DCM patients have familial DCM; up to 40% of whom have an identifiable genetic cause<sup>(1)</sup>
- Scientific societies recently endorsed clinical genetic testing for DCM patients and families<sup>(2,3)</sup>
- Prevalence of BAG3 DCM in US estimated to be as high as 30,000 patients<sup>(4)</sup> and is expected to grow with increasing genetic testing and disease awareness

Virani et al., Circ. 2021; Steinberg et al., Circ. 2012; Brouwers et al., Eur Heart J. 2013; Bhambhani et al., Eur J Heart Fail. 2018; Kapoor et al., JACC:Heart Fail. 2016; Pfeffer et al., Lancet 2003; Balmforth et al., JACC:Heart Fail. 2019; Felker et al., Properties et al., Eur J Heart J. 2015; Kindel et al., Jacc Et al., Jacc Et al., Genet Med. 2014; Petretta et al., Am J Cardiol. 2011; McNally and Mestroni, Circ Res. 2017; Sweet et al., Exp. Op. Orphan Drugs 2016; Ganesh et al., Circ. 2013; Aragam et al., AHA Scient. Sess. 2021; Villard et al., Eur. Heart J. 2011; Franszczyk et al., J Trans Med. 2014; Chami et al., Can J Cardiol. 2014; Arimura et al., Human Mut. 2011; Dominguez et al., JACC 2018; Norton et al., Am J Human Gen. 2011.



American Heart Association Statistical Update: Heart Disease and Stroke Statistics – 2022, mortality rate includes myocarditis.

Ackerman MJ et al. Heart Rhythm 2011

Musumuru K et al. Circulation: Genomic and Precision Medicine 2020.

# Rocket Pharmaceuticals: Elevating Gene Therapy to New Heights

#### WHERE WE ARE



- Recognized as a premier gene therapy company
- Specialized in monogenic diseases
- Pioneer in the development of both ex vivo LV and in vivo AAV therapies
- AAV9-based gene therapy for Danon disease, a major value driver based on size of indication and lack of other therapies
- LV-based programs to provide near term commercialization

#### WHERE WE'RE GOING



- Commercial company with initial therapies and revenue build for FA, LAD-I and PKD, plus leading cardiac franchise led by Danon disease followed by BAG3 DCM
- Broad pipeline of additional new Wave 2 therapies targeting potentially larger opportunities
- Potential new technologies employed



# Strengthen Balance Sheet with Cash Runway Extended into 2Q'24

# Current cash runway into 1H24

Cash and Cash
 Equivalent on hand of
 \$321.4 million as of
 6/30/22

# Extend cash runway into 2Q'24

- \$46 million in net ATM proceeds to date
- Expected to add \$38M in projected net cash at RCOR closing by 1Q23

# Additional alternatives to extend cash runway further

- Consider options of private / public financings
- Two PRVs may be used for monetization upon FA/LAD-I FDA approvals in 1H24



### Conclusion & Expected Next Steps

#### ROCKET'S LEADING CARDIAC GENE THERAPY FRANCHISE HAS A PIVOTAL YEAR AHEAD

- Phase 1 pediatric cohort data in Danon expected in Q3; opportunity for strong validation of Rocket's efforts in cardiac AAV gene therapy
- Initiation of Phase 2 pivotal trial
- Preclinical data and updates on clinical development pathway in BAG3-DCM anticipated in near term
- Potential for Phase 1 initiation in BAG3-DCM in 2023

#### Near term inflection points drive value

2022

Q2

Q3

**Q4** 

2023

- Achieve in-house AAV (cGMP) readiness
- ✓ LAD-I: Pivotal Phase 2 data
- **FA**: Phase 2 primary endpoint readout

Danon: Phase 1
 pediatric cohort data

**Danon**: FDA EOP1 agreement and initiation of Phase 2 pivotal study activities

- FA: Potential guidance on BLA filing timeline
- PKD: Phase 1 data

- LAD-I: BLA/MAA filings 1H'23
- FA: BLA/MAA filings in 2023
- **PKD**: Initiate Phase 2 pivotal trial activities 1H'23
- Wave 2 (including BAG3-DCM) pipeline enters clinic



#### **Forward-Looking Statements**

This communication relates to a proposed business combination transaction between Rocket Pharmaceuticals, Inc. ("Parent") and the Company (the "Company"). This communication includes forward-looking statements within the meaning of Section 27A of the Securities Act of 1933 and Section 21B of the Securities Exchange Act of 1934. Forward-looking statements relate to future events and anticipated results of operations, business strategies, the anticipated transaction, the anticipated impact of the proposed transaction and to the rope of the proposed transaction, the anticipated closing date for the proposed transaction and other aspects of our operations or operating results. These forward-looking statements generally can be identified by phrases such as "will," "should," "expects," "plans," "anticipates," "could," "intends," "target," "projects," "contemplates," "believes," "predicts," "porticula," "contending," "developed and celeration of the events anticipated by the forward-looking statements will transpire or occur, or if any of them do, what impact they will have on the results of operations and financial condition of the companies or the price of Parent's or the Company's stock. These forward-looking statements involve certain risks and uncertainties, many of which are beyond the parties' control, that could cause actual results to differ materially from those indicated in such forward-looking statements, including but not limited to the impact of public health crises, such as pandemics (including coronavirus (COVID-19)) and epidemics and any related company or government policies and actions to protect the health and safety of individuals or government policies and actions to protect the health and safety of individuals or government policies or actions to maintain the functioning of national or global economics and markets; the effect of the announcement of the merger on the ability of Parent or the Company's product candidates; the risk that the merger districts of participation in each of Parent's on g

#### No Offer or Solicitation

This communication is not intended to and shall not constitute an offer to buy or sell or the solicitation of an offer to buy or sell any securities, or a solicitation of any vote or approval, nor shall there be any sale of securities in any jurisdiction in which such offer, solicitation or sale would be unlawful prior to registration or qualification under the securities laws of any such jurisdiction. No offering of securities shall be made, except by means of a prospectus meeting the requirements of Section 10 of the U.S. Securities Act of 1933, as amended.

#### Additional Information about the Merger and Where to Find It

n connection with the proposed transaction, Parent intends to file with the SEC a registration statement on Form S-4 that will include a proxy statement of the Company and that also constitutes a prospectus of Parent. Each of Parent and the Company may also file other relevant documents with the SEC regarding the proposed transaction. This document is not a substitute for the proxy statement/prospectus or registration statement or any other document that Parent or the Company may file with the SEC. The definitive proxy statement/prospectus (if and when available) will be mailed to stockholders of Parent and the Company. INVESTORS AND SECURITY HOLDERS ARE URGED TO READ THE REGISTRATION STATEMENT, PROXY STATEMENT/PROSPECTUS AND ANY OTHER RELEVANT DOCUMENTS THAT MAY BE FILED WITH THE SEC, AS WELL AS ANY AMENDMENTS OF SUPPLEMENTS TO THESE DOCUMENTS, CAREFULLY AND IN THEIR ENTIRETY IF AND WHEN THEY BECOME AVAILABLE BECAUSE THEY CONTAIN OR WILL CONTAIN IMPORTANT INFORMATION ABOUT THE PROPOSED TRANSACTION. Investors and security holders will be able to obtain free copies of the registration statement and proxy statement/prospectus (if and when available) and other documents containing important information about Parent, the Company and the proposed transaction, once such documents are filed with the SEC through the website maintained by the SEC at <a href="https://www.sec.gov">https://www.sec.gov</a>. Copies of the documents filed with the SEC by Parent will be available free of charge on the Parent's website at <a href="https://ir.renovacor.com">https://ir.renovacor.com</a> or by contacting the Company's Investor Relations department at <a href="morenovacor.com">investors@renovacor.com</a>. Copies of the documents filed with the SEC by the Company will be available free of charge on the Company's website at <a href="https://ir.renovacor.com">https://ir.renovacor.com</a> or by contacting the Company's Investor Relations department at <a href="morenovacor.com">investors@renovacor.com</a>. Copies of the documents filed

#### Participants in the Solicitation

Parent, the Company and certain of their respective directors and executive officers may be deemed to be participants in the solicitation of proxies in respect of the proposed transaction. Information about the directors and executive officers of Parent and the Company, including a description of their direct or indirect interests, by security holdings or otherwise, is set forth in Parent's and the Company's Annual Reports filed with the SEC on April 29, 2022 and April 14, 2022, respectively. Other information regarding the participants in the proxy solicitations and a description of their direct and indirect interests, by security holdings or otherwise, will be contained in the proxy statement/prospectus and other relevant materials to be filed with the SEC regarding the proposed transaction when such materials become available. Investors should read the proxy statement/prospectus carefully when it becomes available before making any voting or investment decisions. You may obtain free copies of these documents from Parent or the Company using the sources indicated above.



# **THANK YOU!**

