

# **Amicus Launches Next-Generation Genetic Medicine Company**

Conference Call & Webcast



September 29, 2021

### Forward-Looking Statements

This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to the business combination, development of Caritas' product candidates, preclinical and clinical development of our product candidates, the timing and reporting of results from preclinical studies and clinical trials, the prospects and timing of the potential regulatory approval of our product candidates, commercialization plans, manufacturing plans and financing plans. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans or Caritas' plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, with respect to statements regarding the goals, progress, timing, and outcomes of discussions with regulatory authorities, and in particular the potential goals, progress, timing, and results of preclinical studies and clinical trials, and revenue goals, including as they are impacted by COVID-19 related disruption, are based on current information. The potential impact on operations and/or revenue from the COVID-19 pandemic is inherently unknown and cannot be predicted with confidence and may cause actual results and performance to differ materially from the statements in this release, including without limitation, because of the impact on general political and economic conditions, including as a result of efforts by governmental authorities to mitigate COVID-19, such as travel bans, shelter in place orders and third-party business closures and resource allocations, manufacturing and supply chain disruptions and limitations on patient access to commercial or clinical product or to treatment sites. In addition to the impact of the COVID-19 pandemic, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in our business, including, without limitation: the potential that the transaction may not be completed in a timely manner or at all, which may adversely affect the price of our securities; the potential that the proposed transaction disrupts our current plans and operations and potential difficulties in our employee retention as a result of the transaction; the potential that results of clinical or preclinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in clinical trials; the potential that regulatory authorities, including the FDA, EMA, and PMDA, may not grant or may delay approval for our product candidates; the potential that Caritas may not be successful in maintaining or establishing collaborations, which could adversely affect its ability to develop products; substantial competition in the rapidly evolving field of gene therapy, which may result in others discovering, developing or commercializing products before or more successfully than Caritas; the potential that regulatory authorities approve biosimilar products with claims that compete with any of Caritas' product candidates; the potential that preclinical and clinical studies could be delayed due to the identification of serious side effects or other safety issues; the potential that we or Caritas may not be able to manufacture or supply sufficient clinical or commercial products; the potential that Caritas may become involved in product liability lawsuits and in lawsuits to protect or enforce intellectual property rights, which could be expensive and time-consuming, and could result in substantial liabilities and the impairment of commercialization efforts of product candidates; and the potential that we or Caritas will need additional funding to complete all of our respective studies, commercialization and manufacturing. In addition, all forward-looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2020 and Quarterly Report 10-Q for the guarter ended June 30, 2021. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise or update this news release to reflect events or circumstances after the date hereof.

#### Disclaimer

#### **Participants in the Solicitation**

ARYA İV and its directors, executive officers, other members of management, and employees, under SEC rules, may be deemed to be participants in the solicitation of proxies of ARYA IV's shareholders in connection with the business combination. Investors and securityholders may obtain more detailed information regarding the names and interests in the business combination of ARYA IV's directors and officers in ARYA IV's filings with the SEC, including ARYA IV's final prospectus which was filed with the SEC pursuant to Rule 424(b) of the Securities Act of 1933, as amended (the "Securities Act") on February 19, 2021, as well as in the Registration Statement, which includes the proxy statement of ARYA IV for the business combination. Shareholders can obtain copies of ARYA IV's filings with the SEC, without charge, at the SEC's website at <a href="https://www.sec.gov">www.sec.gov</a>.

#### **No Offer or Solicitation**

This presentation is for informational purposes only and is neither an offer to purchase, nor a solicitation of an offer to sell, subscribe for or buy any securities or the solicitation of any vote in any jurisdiction pursuant to the business combination or otherwise, nor shall there be any sale, issuance or transfer or securities in any jurisdiction in contravention of applicable law. No offer of securities shall be made except by means of a prospectus meeting the requirements of the Securities Act and otherwise in accordance with applicable law.



### **Executive Summary**

- AT-GAA NDA/BLA accepted for review: respective PDUFA action dates of May 29, 2022 and July 29, 2022
- Amicus gene therapy programs, technologies, IP, and relationships to form new company: Caritas Therapeutics
- Caritas to be acquired by ARYA IV, a special purpose acquisition company, and launch with ~\$400M in capital (through SPAC, PIPE and Amicus funding)
- Caritas immediately to become one of the world's preeminent next-generation gene therapy and genetic medicines company
- John Crowley will lead Caritas as Chairman and CEO and to remain with Amicus as Chairman Emeritus and Chief Strategic Advisor
- Bradley Campbell to succeed John Crowley as Amicus Chief Executive Officer upon transaction closing
- Amicus expects to become profitable as of 2023
- Amicus further funded through completed \$200M private investment







## A RARE COMPANY

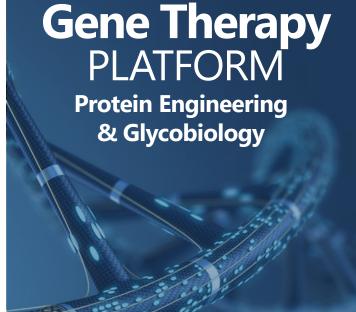
#### Amicus has built a leading fully integrated, global rare disease biotechnology company



First Oral Precision Medicine for Fabry Disease







Robust R&D Engine 50+ Lysosomal Diso

50+ Lysosomal Disorders and More Prevalent Rare Diseases

**World Class** 

**BIOLOGICS** 

**Capabilities** 

**EMPLOYEES**in 27 Countries

**\$383M**Cash
as of 6/30/21





GLOBAL COMMERCIAL ORGANIZATION



### Operating from Position of Strength

#### Two lead programs each with potential \$500M - \$1B peak sales opportunity

Galafold® continues to exceed expectations in Fabry disease

AT-GAA submissions in Pompe disease are on track to kick off launch next year









- NDA & BLA accepted for review
- PDUFA action dates of May 29, 2022 and July 29, 2022
- EU MAA submissions to be completed in Q4 2021
- UK submission via recognition procedure based on CHMP opinion



#### Poised to Unlock Value

Significant but unrecognized value created across Amicus' leading rare disease gene therapy pipeline and cutting-edge next-generation platform technologies and partnerships



<sup>\*</sup>Caritas Therapeutics will have rights to gene therapy technologies for the majority of LSDs, plus 11 additional larger Rare Diseases, including Angelman Syndrome, Duchenne Muscular Dystrophy (DMD), Rett Syndrome, Myotonic Dystrophy and select other muscular dystrophies

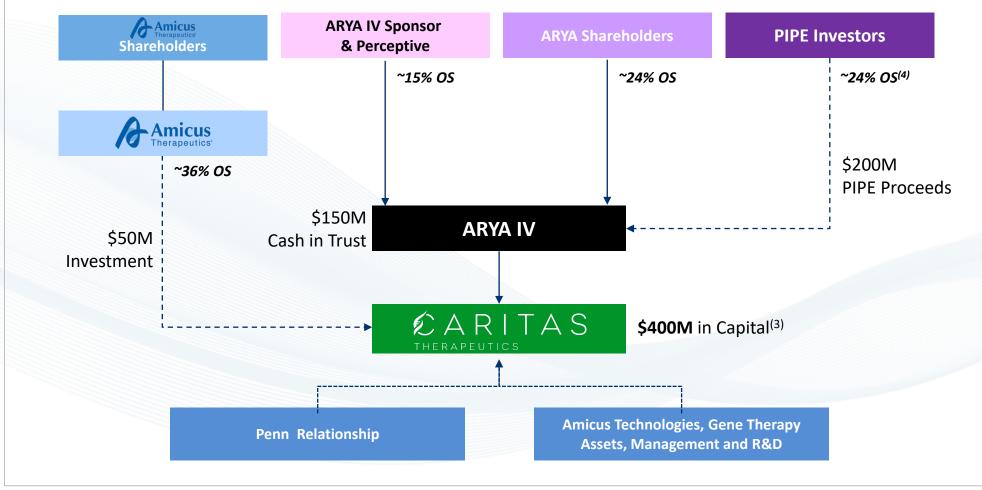


### Simplified Transaction Structure<sup>(1)</sup>

#### Amicus intends to launch Caritas as a next-generation genetic medicine company

- Combining Amicus's gene therapy business with ARYA IV, a special purpose acquisition company (SPAC)
- Caritas is carved out of Amicus, retaining a collaboration with the Perelman School of Medicine at the University of Pennsylvania (Penn) as well as forming a new collaboration with Amicus
- Amicus retains an interest in the combined entity (~36% pro forma ownership)<sup>(2)</sup>
- The combined entity, renamed Caritas, is capitalized with \$400M to execute on its R&D plan: up to \$150M from ARYA IV's trust, as well as an expected \$200M from leading PIPE investors, and a \$50M Amicus cash investment

Transaction Structure: Carving out Caritas and Combining with ARYA IV<sup>(2)</sup>



(1) Assumes no shareholder redemptions and based on implied share price of \$10 per share; (2) OS percentages show indirect ownership in Caritas without regard to equity incentive and other awards and includes a \$50M investment from Amicus as contemplated by BCA (assuming no shareholder redemptions investment may increase to serve as backstop against redemptions); (3) Excludes transaction fees and expenses; (4) Excludes PIPE commitment from Perceptive



### Strategic Rationale

We believe this transaction will generate greater value for all stakeholders, significant therapeutic advancement, and result in benefits to the stand-alone companies

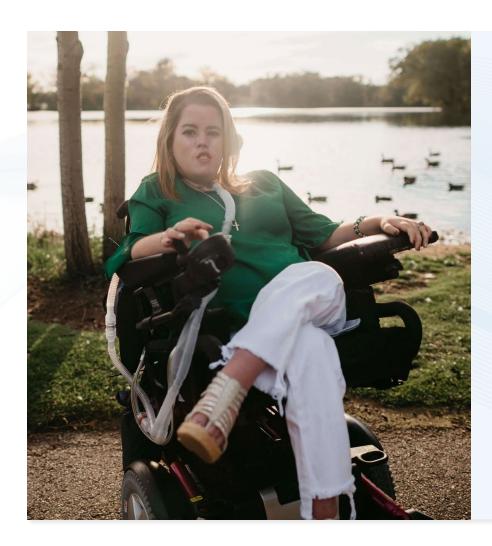


- 2 Accelerating Amicus path to profitability, expected in 2023
- 3 Significantly strengthening the financial profile of each company
- Accelerating the development and broadening the scope of our gene therapy portfolio
- Reinforcing management focus on key strategic and financial goals
- 6 Unlocking value while creating a more targeted investment thesis for shareholders



### Transforming for the Future

Transaction transforms Amicus into a premier later stage development and commercialization company and builds upon its leadership in Fabry and Pompe diseases



- Amicus remains a global, patient-dedicated biotechnology company developing and delivering novel high-quality medicines for people living with rare diseases
- Focus will be to grow its leadership position across the Company's two lead indications of Fabry disease and Pompe disease:
  - Continue investing in the global commercialization of Galafold in Fabry disease through continued geographic and label expansion, as well as support for diagnostic initiatives
  - Focus on securing global approvals and executing the anticipated global launch of AT-GAA in Pompe disease
  - Prepare for co-development of gene therapy programs with Caritas in Fabry and Pompe diseases



### Strengthening the Financial Profile of Amicus

Dramatically strengthens Amicus financial profile while preserving upside in gene therapy portfolio of programs and platform technologies

- 1 Increases focus on commercial success of Galafold and AT-GAA
  - Aims at maximizing patient access to Galafold and AT-GAA, each with \$500M \$1B peak sales opportunity
- Strengthens the financial outlook of Amicus
  - Reiterating financial guidance for 2021
  - Transferring significant R&D expenses into Caritas
  - Cash position increased through private investment of leading healthcare investors
  - Amicus becomes profitable in 2023
- Preserves upside in gene therapy programs and platform technologies
  - Secures 36% pro forma equity ownership in the new entity, Caritas
  - Joint development and exclusive global commercial rights to both the Fabry and Pompe gene therapies, including 50/50 profit share
  - Right of first negotiation to multiple muscular dystrophy programs being developed by Caritas



### Harnessing the Power of Genetic Medicine

The mission of Caritas is to transform the lives of children and adults living with rare genetic diseases through advanced protein engineering and innovative vector technologies



- Proprietary platform technologies and protein engineering capabilities enabling innovative, clinically differentiated gene therapies
  - **Exclusive relationship with Penn to develop gene therapies for rare genetic disorders**

**EARITAS** 

- Fully designed, ready-to-build, state-of-the-art clinical manufacturing facility with commercial expansion capabilities
- Attractive risk and cost-sharing partnership with Amicus provides access to an established orphan disease commercial platform
- Experienced public company leadership coupled with fully built out gene therapy discovery, research and development team



### Three Strategic Value Drivers

#### Deep science in protein engineering driving a robust pipeline supported by industry-leading partnerships

Science

- Modern protein engineering platforms generating bespoke solutions
- Engineered proteins and transgenes for enhanced expression, cross correction, targeting and immune protection

EARITAS THERAPEUTICS

**Pipeline** 

- 2 Batten disease programs with clinical proof of concept
- 6 active preclinical programs, including Fabry disease, Pompe disease, CDD
- Multiple discovery programs, including Angelman Syndrome

**Partnerships** 

- Penn collaboration with exclusive rights to next-generation AAV technologies for named indications
- Strategic relationship with Thermo Fisher for manufacturing
- Amicus co-development and commercialization agreement (Fabry & Pompe GTx)





### Co-Development and Commercialization Agreement

#### New Amicus and Caritas collaboration around genetic medicine for Fabry and Pompe diseases

- Differentiated gene therapy approach for greater potency and optimized cross correction through transgene engineering for stability and targeting
  - Fabry disease: IND expected in 2H2022
  - Pompe disease: IND enabling work underway
- Partnering with experts in the Fabry and Pompe field and securing key long-term relationship
- De-risks funding for Fabry and Pompe gene therapy programs with
   50% funding split to commercialization and a net profit split on sales
- Potential for other disease collaborations given Amicus' right of first negotiation for muscular dystrophies



### Planning for a Seamless Evolution in Leadership







John F. Crowley
CHAIRMAN & CHIEF EXECUTIVE OFFICER

Bradley Campbell
PRESIDENT & CHIEF OPERATING OFFICER

- Amicus Chairman & CEO for 15+ years
- Co-founder, President and CEO of Novazyme Pharmaceuticals (acquired by Genzyme in 2001)
- Member of the Intellia and Entrada Therapeutics Board of Directors
- B.S. from Georgetown University, J.D. from the University of Notre Dame Law School and an M.B.A. from Harvard Business School
- **○** Upon completion of the separation, John F. Crowley will lead Caritas as Chairman and CEO
- **○** John F. Crowley will be Chairman Emeritus of Amicus and employed as Chief Strategic Advisor to Amicus

- Amicus Executive for 15+ years, President & COO since 2014,
   Director since 2019
- Member of the Board of the Association for Regenerative Medicine (ARM) and the Corporate Advisory Board for NTSAD
- Served on the Board of Progenics Pharmaceuticals from 2016 until its successful acquisition by Lantheus Holdings in 2020
- B.A. in Public Policy from Duke University and an M.B.A. from Harvard Business School
- **○** Upon completion of the separation, Bradley Campbell will be named CEO of Amicus



### Transaction Summary

#### Unlocking value by allowing two stand-alone companies to realize their full potential

## **Transaction Details**

- Business combination of the Amicus Gene Therapy Business with ARYA IV, a SPAC, resulting in the launch of Caritas Therapeutics, an independent, publicly traded company
- Amicus to own ~36% in Caritas, retain co-development and commercial rights to the Fabry and Pompe gene therapies including 50/50 profit share, and right of first negotiation to multiple muscular dystrophy programs being developed at Caritas

## **Strategic Rationale**

- Enhancing the ability of both companies to meet the unmet needs of many individuals and families affected by rare diseases
- Accelerating the development and broadening the scope of the gene therapy portfolio
- Strengthening the financial profile of each company
- · Reinforcing management focus on key strategic and financial goals
- Unlocking value while creating a more targeted investment thesis for shareholders

#### Leadership

- John F. Crowley, current Chairman and CEO of Amicus, to be Chairman and CEO of Caritas; will be Chairman Emeritus of Amicus and employed as Chief Strategic Advisor to Amicus
- Bradley Campbell, current President and COO of Amicus, to be CEO of Amicus

## Financial Amicus Therapeutics Details Amicus Therapeutics

- Balance sheet strengthened with \$200M private investment; profitability expected as of 2023
- 2021 financial guidance unchanged
- SPAC Trust, PIPE and Amicus investment to provide \$400M of proceeds funding Caritas through multiple value-creating milestones

#### **Timing**

Completion of the transaction is expected in late 2021/early 2022, subject to approval of ARYA IV's shareholders and the satisfaction or waiver of certain other customary closing conditions



## Thank You

"Our passion for making a difference unites us"
-Amicus Belief Statement

