

# Leveraging a patient's own biology to deliver rare disease therapeutics

The Citizens JMP Life Sciences Conference May 13, 2024

## **Forward-looking statements**

Statements in this presentation contain "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 as contained in Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, which are subject to the "safe harbor" created by those sections. All statements, other than statements of historical facts, may be forward-looking statements. Forward-looking statements contained in this news release may be identified by the use of words such as "believe," "may," "should," "expect," "anticipate," "plan," "believe," "estimated," "potential," "intend," "will," "can," "seek," or other similar words. Examples of forward-looking statements include, among others, statements relating to current and future clinical development of EryDex, including for the potential treatment of Ataxia-Telangiectasia (A-T), Duchenne muscular dystrophy (DMD), and other potential indications, related development and commercial-stage inflection point for EryDex, and expansion of the company's proprietary Autologous Intracellular Drug Encapsulation (AIDE) technology for treatment of other rare diseases; the strategic development path for EryDex; planned regulatory agency submissions and clinical trials and timeline, prospects, and milestone expectations; the timing and success of the clinical trials and related data, including plans and the ability to initiate, fund, enroll, conduct, and/or complete current and additional studies; research and development costs; the company's future development plans and related timing; cash position and projected cash runway; the company's focus, objectives, plans, and strategies; and the potential benefits of EryDex, AIDE technology, and the company's market opportunity. Forward-looking statements are based on Quince's current expectations and are subject to inherent uncertainties, risks, and assumptions that are difficult to predict and could cause actual results to differ materially from what the company expects. Further, certain forward-looking statements are based on assumptions as to future events that may not prove to be accurate. Factors that could cause actual results to differ include, but are not limited to, the risks and uncertainties described in the section titled "Risk Factors" in the company's Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) on April 1, 2024, and other reports as filed with the SEC. Forward-looking statements contained in this presentation are made as of this date, and Quince undertakes no duty to update such information except as required under applicable law.



# **Quince Therapeutics investment highlights**



Acquisition closed on October 20, 2023



## Completed transformative acquisition of EryDel S.p.A.

Rare disease focused with compelling Phase 3 lead asset, EryDex, for potential treatment of Ataxia-Telangiectasia (A-T) with no currently approved treatments and \$1+ billion\* peak commercial opportunity globally

## Phase 3 lead asset EryDex enrollment planned for second quarter 2024

Risk-mitigated clinical and regulatory approach supported by optimized clinical trial design, special protocol assessment (SPA) agreement with FDA, and encouraging Phase 3 clinical trial results in prior study

## Strong balance sheet to achieve meaningful clinical inflection point

Well-capitalized with sufficient cash runway into 2026 expected to fund EryDex through Phase 3 topline results expected in second half of 2025 and prepare for a potential NDA and MAA submissions in 2026, assuming positive study results

# Unique drug/device combination with high barriers to entry

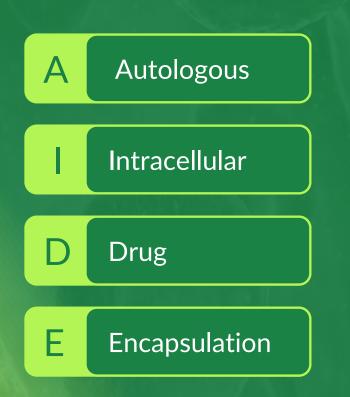
- One-touch, fully automated, and sterile Autologous Intracellular Drug Encapsulation (AIDE) device
  - Designed to deliver therapeutic in patient's own red blood cells – distinct from standard cell or gene therapy
- Flexible technology designed to deliver wide range of therapeutics from small and large molecules to biologics
- CE mark in Europe with strong patent protections and IP exclusivity until at least 2034 globally & 2035 in U.S. – without patent term adjustment or extension



20+ years of research & development and \$100 million invested in AIDE technology



Unlocking the potential of a patient's own biology to deliver innovative and life-changing therapeutics to those living with rare diseases



# **Proprietary AIDE technology process**

> Patient's own blood collected and loaded into device using consumable treatment kit for fully automated and sterile processing

AIDE processes red blood cells to encapsulate therapeutic of interest – dexamethasone sodium phosphate (DSP) in case of lead asset, EryDex

Result of the process is DSP loaded red blood cells that are washed, isolated, and prepared – no gene editing or conditioning regimen required

- Proprietary process results in DSP encapsulated in autologous red blood cells that is then infused into patient
- Approximately two-hour process designed for monthly outpatient administration, if approved

Designed to fundamentally alter biodistribution and pharmacokinetics of DSP to allow for sustained therapy – mitigating chronic toxicity and adrenal suppression associated with long-term steroid use

#### **EryDex designed to optimize dexamethasone delivery EryDex** through once monthly dosing in A-T patients ATTeST Trial **Completed largest** global study of A-T Mean concentration-time profile for plasma dexamethasone in EryDex patients in Phase 3 ATTeST clinical trial (N=175) over 1,000 6-month treatment period with 12-Mean plasma concentration month OLE (N=104) 100 **ErvDex pharmacokinetic profile designed to deliver** 10 adequate Cmax followed by gradual taper and continuous exposure to maintain glucocorticoid receptors occupancy (ng/mL) sustained over entire month 1 0.1 0.01 **Sustained Efficacy Window** 0.001 5 10 15 20 25 0 30 Days 17.4 mg is dosage used in pivotal **Phase 3 NEAT study** Mean EryDex monthly dose = 17.4 mg

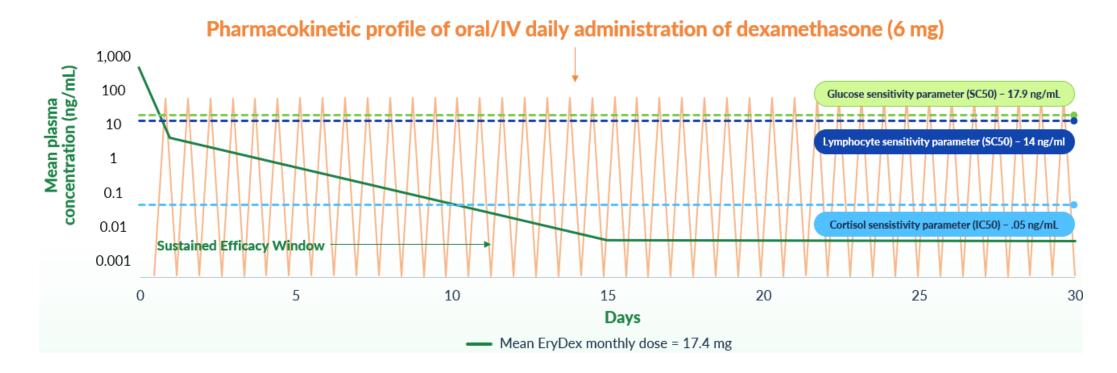
## Why are conventional corticosteroids toxic?

## Well-described dose-limiting toxicity of corticosteroids

#### Adrenal suppression $(\mathbf{X})$

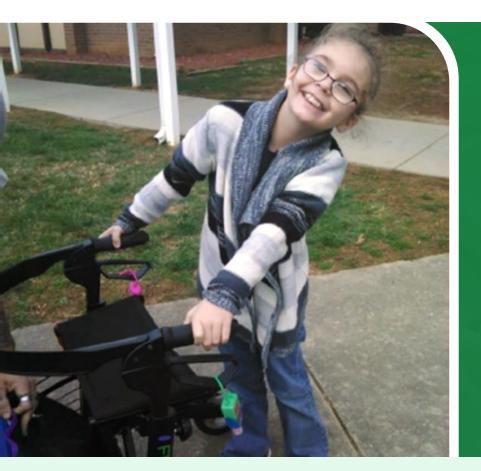
- Low cortisol levels
- Cushingoid appearance
- Hirsutism
- Loss of bone mineral density
- Growth retardation in pediatrics
- Delay in puberty

- **Elevated glucose resulting in**  $(\mathbf{X})$ hyperglycemia and diabetes
- Immunosuppression resulting  $(\mathbf{X})$ in infections



Note: Information represented does not reflect a completed comparative study of EryDex versus oral/IV administration of dexamethasone, but rather provides a comparison of published corticosteroid pharmacokinetic information relative to company data regarding EryDex. IC50 and SC50 refer to pharmacodynamic parameters of which IC50 reflects drug concentration eliciting 50% of the maximum inhibition and SC50 reflects drug concentration eliciting 50% of the maximum stimulation. References: Company prior Phase 3 ATTeST clinical trial data (ClinicalTrials.gov ID: NCT02770807); Montanha et al, Frontiers in Pharmacology (2022) 13: 814134; Krzyzanski et al, Journal of Pharmacokinetics and Pharmacodynamics (2021) 48: 411-438; Aljebab et al, PLOS ONE (2017) 10: 1371.

# No currently approved treatments for A-T patients



A-T is an inherited rare neurodegenerative and immunodeficiency disorder caused by mutations in ATM gene

 Estimated prevalence of approximately 10,000 patients with A-T in U.S., U.K., and EU4 countries\*

Neurological symptoms worsen until patients are wheelchair dependent, usually by age 12 – with teenage years typically marked by repeated infections, pulmonary impairment, and malignancies

Median lifespan of approximately 25-30 years

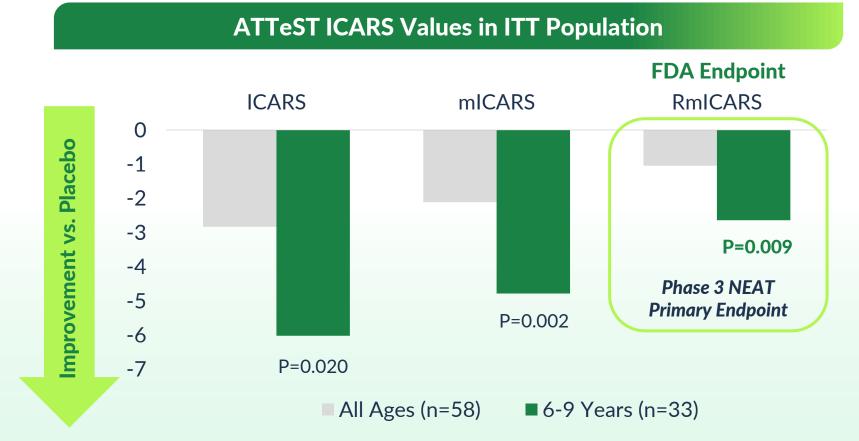
Currently no approved treatments for A-T and no currently known effective approaches to delay progression of disease



# Encouraging EryDex Phase 3 clinical trial results in prior ATTeST study



- Improvement observed in
   6 to 9 year-old subgroup
   across multiple endpoints
- At 12 months, EryDex well-tolerated with no serious safety concerns
- 3+ years of ATTeST OLE observed no serious safety concerns
- Apply key learnings from ATTeST to pivotal NEAT study



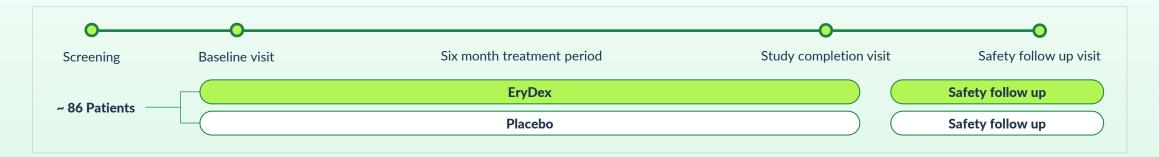


Note: Company ATTeST clinical trial data (ClinicalTrials.gov ID: NCT02770807) presented reflect nominal p-values for ICARS values in ITT populations. Values reflect Least Square Means (LSM) difference from placebo and the P value presented • ICARS = International Cooperative Ataxia Rating Scale • mICARS = Modified International Cooperative Ataxia Rating Scale • mICARS = Rescored Modified International Cooperative Ataxia Rating Scale

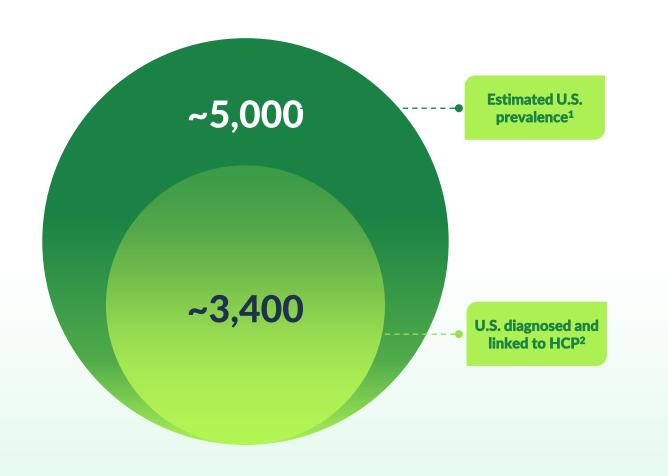
## **Pivotal Phase 3 NEAT study design**

- Pivotal study to be conducted under Special Protocol Assessment (SPA) agreement with FDA Allows for NDA submission, assuming positive results, following a single global Phase 3 NEAT study
- Randomized, double-blind, placebo-controlled study with six infusions scheduled once every 21 to 30 days Plan to enroll first patient in global Phase 3 NEAT clinical trial of EryDex in second quarter 2024
- Plan to enroll approximately 86 patients with A-T ages 6 to 9 years old (primary analysis population) Approximately 20 additional patients with A-T ages 10 years or older also will be included Participants will be eligible to transition to an open label extension (OLE) study

## > Primary efficacy endpoint - RmICARS



# A-T represents significant U.S. commercial opportunity



Approximately 3,400 diagnosed U.S. patients with A-T

No currently approved A-T treatments, potential to expand patient number with market development

 Benefit from engaged, connected A-T patient community

# Attractive commercial opportunity for EryDex lead indication

# **\$1+ billion**\*

estimated global peak commercial opportunity for A-T indication alone

## **EryDex for A-T**

Sestimated prevalence of approximately 10,000 patients with A-T\* in U.S., U.K., and EU4 countries with no currently approved A-T therapies

First-to-market potential with no known late-stage competition and granted orphan drug designation for the treatment of A-T from FDA and EMA

Attractive rare disease pricing comparables with recently approved treatment for Friedreich's ataxia indication

Highly scalable manufacturing infrastructure in place with low direct cost of goods – less than 1% of comparable U.S. rare disease pricing

European/U.S. orphan drug designation and strong patent protections with IP exclusivity until at least 2034 globally and 2035 in the U.S. – without patent term adjustment or extension



\*\$1+ billion estimated global peak commercial opportunity and estimated A-T patient population are based on IQVIA Medical Claims (Dx), IQVIA Analytics in the U.S. and the company's internal estimates and assumptions outside the U.S.

# **Expansion potential of EryDex and AIDE technology into additional rare disease indications**

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EryDex system for indications beyond A-T where chronic steroid treatment is – or has the potential to become – a standard of care

Selected Duchenne muscular dystrophy (DMD) as second development program – ideal indication given well described clinical benefits of steroids in patients with DMD

Generating proof-of-concept clinical trial study designs to evaluate EryDex for the potential treatment of patients with DMD, including corticosteroid intolerant populations

Continuing investigating other potential indications for EryDex spanning across ataxias, neuromuscular indications, hematology, cancer, and autoimmune diseases, with a focus on rare diseases

Rare and debilitating disease expansion potential

Flexible technology designed to deliver range of therapeutics – from small and large molecules to biologics

Evaluation of additional potential applications of AIDE technology platform for new rare and debilitating disease programs already underway

E

Α

EryDex

Autologous

Intracellular

**Encapsulation** 

Drug

# **Corticosteroids have** application across many diseases

EryDex holds potential to expand into clinical proof of concept for broad range of rare disease indications



## Neurology/Neuromuscular

 $\odot$  Ataxia telangiectasia (1<sup>st</sup> indication)

**⊘** Duchenne Muscular Dystrophy (2<sup>nd</sup> indication)



Rheumatology



Endocrinology

Dermatology

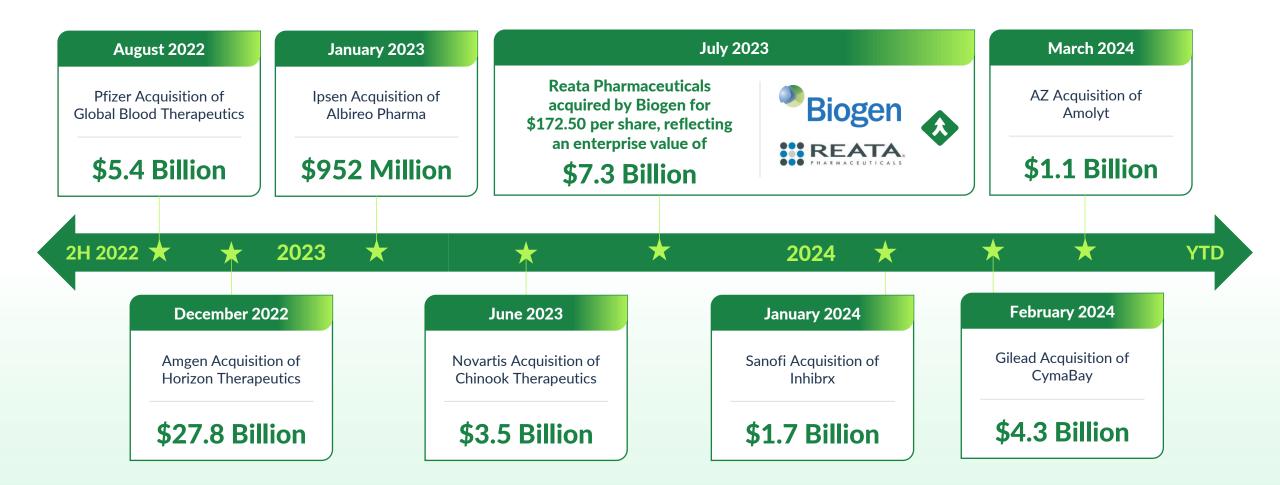




Inflammatory



## **Recent transaction activity in rare disease space**



Additional ~\$7 billion committed in licensing and collaboration deals within rare disease space in 2023 alone

# **Seasoned leadership team**



CEO & CMO

### Dirk Thye, M.D.

- 20+ years of experience in biotech executive leadership, company creation, R&D, and drug discovery
- Agenovir, Cidara, Cerexa, Peninsula



& PFO

## Brendan Hannah, M.B.A.

- 15+ years leading biotech BD, finance, and business operations
- Led BD at Agenovir (acquired by Vir Biotech for up to \$290 million)
- Involved in \$2+ billion in transactions



## Thomas Sabia, M.B.A.

- 20+ years of drug commercialization and development experience across large, midsize, and small biotech organizations
- Spark Therapeutics (a Roche Company), Sobi, CSL Behring, Sanofi



### Giovanni Mambrini, MSc

- 20+ years of medical device technology experience with crossfunctional expertise in bringing complex programs to market
- Co-founded EryDel with prior experience at Covidien, Dideco, Livanova



## Charles Ryan J.D., Ph.D.

- 25+ years of experience in pharmaceuticals and biotech executive leadership, legal, IP, finance, and development
- Forest Laboratories, Travecta, Neurotrope



## Pamela Williamson, RAC, FRAPS, M.B.A.

• 30+ years of regulatory affairs, quality assurance, pharmacovigilance, health authority compliance and manufacturing operations experience



Alexion Pharmaceuticals, Genzyme Corporation, Serono/Ares-Serono



## Maureen Roden, M.S.N.

- 30+ years of drug development experience and executive leadership
- Luna Consulting, BSPI, National Cancer Institute

## VP, Clin Dev



## Gary Ward, Ph.D.

- 30 + years managing chemistry, manufacturing, and controls and product development operations
- Expert in broad range of NCE types/product dosage forms
- Pfizer, Dura Pharma, Chugai Biopharma, 3M Company





Strong balance sheet with approximately \$67.8 million in cash, cash equivalents, and short-term investments as of March 31, 2024

Well-capitalized with sufficient cash runway into 2026



- EryDex through Phase 3 NEAT topline results and preparing for potential NDA and MAA submissions, assuming positive study results
- NEAT study and OLE direct trial costs of ~\$20 million and ~\$15 million, respectively
- Investigation of other potential indications for EryDex
- Evaluation of additional potential applications of AIDE technology for new rare and debilitating diseases



**Evaluate potential strategic partnerships to out-license of ex-U.S. rights to provide additional operating runway** 



# Key clinical and corporate milestones

2024	2025
Initiation of Phase 3 NEAT clinical trial enrollment	S Completion of Phase 3 NEAT study enrollment
Selected Duchenne Muscular Dystrophy (DMD) as second indication for EryDex	> Phase 3 NEAT clinical trial topline results
Senerate study designs to evaluate EryDex for the potential treatment of patients with DMD	Prepare for potential NDA and MAA submissions in 2026, assuming positive study results
Determine other potential indications for EryDex and initiate R&D activities	<ul> <li>Initiate at least one new clinical study for additional EryDex indication (e.g. DMD)</li> </ul>
Initiation of Phase 3 NEAT open label extension	Potential out-licensing of ex-U.S. regional territories to provide runway through approval

