

Unlocking the power of a patient's own biology for the treatment of rare disease

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### **Quince Therapeutics investment highlights**

Risk-mitigated pivotal Phase 3 clinical trial of EryDex underway to evaluate lead rare disease asset



EryDex targets Ataxia-Telangiectasia (A-T) with no currently approved treatments and \$1+ billion commercial opportunity



) Selected Duchenne muscular dystrophy (DMD) as second indication for EryDex given high unmet need



) EryDex holds potential to redefine the standard of care for chronic corticosteroid administration without toxicities



Cash runway through pivotal Phase 3 topline results into 2026

# Corticosteroids encapsulated in autologous red blood cells designed to fundamentally alter drug characteristics

Autologous red blood cells (RBCs) ideal vehicle for drug delivery with *potential* for:

- ✓ Unique biodistribution that may enable slow release of drug while circulating through tissues, organs, and capillary beds where therapeutic effect is desired
- ✓ Altered pharmacokinetics and pharmacodynamics, including long circulating half-life, and altered or improved tissue distribution, may significantly increase desired therapeutic effect and/or improve safety profile
- ✓ Improved biocompatibility through use of autologous RBCs, thereby avoiding issues with donor compatibility (no engineered or donor RBCs utilized)
- Mitigating chronic toxicity and adrenal suppression associated with long-term corticosteroid use

Autologous Intracellular Drug Encapsulation (AIDE) Technology



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



## Lead asset EryDex encapsulates dexamethasone sodium phosphate (DSP)



## Why are conventional corticosteroids toxic?

#### Well-described dose-limiting toxicity of corticosteroids

#### × Adrenal suppression

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

- Elevated glucose resulting in hyperglycemia and diabetes
- Immunosuppression resulting 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. References: Montanha et al, Frontiers in Pharmacology (2022) 13: 814134; Świerczek A, Jusko WJ., Clinical and Translational Science (2023) 16(9):1667-1679.

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# **EryDex designed to optimize dexamethasone delivery through once monthly dosing in A-T patients**

Average concentration-time profile for plasma dexamethasone in EryDex



— Average Concentration EryDex monthly dose = 17.4 mg



Note: Pharmacokinetic (PK) curve from Population PK model (smoothed) based on company's prior studies of 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: Montanha et al, Frontiers in Pharmacology (2022) 13: 814134; Krzyzanski et al, Journal of Pharmacokinetics and Pharmacodynamics (2021) 48: 411-438.

### 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



\*Patient population based estimated A-T patient population are based on IQVIA Medical Claims (Dx), PharmetricsPlus (P+), and IQVIA Analytics in the U.S. and the company's internal estimates and assumptions outside the U.S.

### **Rapid neurological progression in A-T**

Symptoms progress quickly from 6 to 9 years of age





\*Scores based on the Crawford Quantitative Neurologic A-T Scale (100=normal). References: Rothblum-Oviatt C, et al. Orphanet J Rare Dis. 2016;11(1):159; Crawford TO, et al. Neurology. 2000;54(7):1505–1509.

# 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 being conducted under Special Protocol Assessment (SPA) agreement with FDA Allows for NDA submission, assuming positive results, following a single global Phase 3 NEAT study
- O Randomized, double-blind, placebo-controlled study with six infusions scheduled once every 21 to 30 days Enrolled first patient in global Phase 3 NEAT clinical trial of EryDex in June 2024 Topline data expected in fourth quarter of 2025

7 patients with A-T enrolled as of mid-August 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

RmICARS measures primarily focused on posture and gait disturbance



## 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 from FDA and EMA, and Fast Track designation from FDA for the treatment of A-T
- Attractive rare disease pricing comparables with recently approved treatment for Friedreich's ataxia indication (Biogen's Skyclarys WAC pricing at \$370K/year)
- Highly scalable manufacturing infrastructure in place with low direct cost of goods less than 1% of comparable U.S. rare disease pricing
- CE mark in Europe and strong patent protections with IP exclusivity until at least 2034 globally and 2035 in U.S. without patent term extension



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



**Physicians expect usage of EryDex in broad A-T Population** 

*Given high unmet need and limited treatment options, HCPs expected utilization across all patient segments* 

|   | Uptake in Ambulatory Patients<br>~90% OF PATIENTS | Uptake in Non-Ambulatory Patients<br>~75% OF PATIENTS |  |
|---|---|---|--|
| • | Physicians were excited by the clinically         | Given limited treatment options, physicians           |  |
|   | meaningful data in ambulatory patients and        | were largely compelled to try EryDex in non-          |  |
|   | expected broad utilization                        | ambulatory patients, despite lack of trial data       |  |
| • | Physicians anticipated use in both classical and  | in this segment                                       |  |
|   | mild patients                                     |   |  |
|   |   |   |  |

### **Selected DMD as second development program for EryDex**

EryDex

For indications beyond A-T where chronic steroid treatment is – or has the potential to become – a standard of care Duchenne muscular dystrophy (DMD) ideal second indication for EryDex 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 in 2024

Potential to start proof of concept study in 2025 in corticosteroid intolerant populations, representing majority of patients with DMD

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



#### **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**





Strong balance sheet with approximately \$59.4 million in cash, cash equivalents, and short-term investments as of June 30, 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
- ~\$20 million NEAT study and ~\$15 million OLE direct trial costs
- Investigation of other potential indications for EryDex, including DMD
- 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   |
|--|--|
| S First patient enrolled in Phase 3 NEAT clinical trial                      | O Completion of Phase 3 NEAT study enrollment  |
| Selected DMD as second indication for EryDex and generating study designs    | > Phase 3 NEAT clinical trial topline results in Q4 2025   |
| Determine other potential indications for EryDex and initiate R&D activities | <ul> <li>Prepare for potential NDA and MAA submissions in 2026, assuming positive study results</li> </ul> |
| Received Fast Track designation for A-T from FDA                             | Initiate DMD clinical study for second EryDex<br>indication  |
| Initiation of Phase 3 NEAT open label extension                              | Potential out-licensing of ex-U.S. regional territories to provide runway through approval                 |
| Phase 3 ATTeST data published in The Lancet Neurology                        |  |

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#### Key investment takeaways

#### Ompelling clinical proposition

- Pivotal Phase 3 clinical trial of EryDex for A-T underway to evaluate lead rare disease asset with topline results expected in fourth quarter of 2025
- Risk-mitigated clinical and regulatory approach supported by optimized clinical trial design, special protocol assessment (SPA) agreement with FDA, Fast Track designation from FDA, and encouraging Phase 3 clinical trial results in prior study

#### > Attractive commercial opportunity

- Lead asset EryDex targets rare disease A-T with no currently approved treatments and \$1+ billion commercial opportunity
- Selected DMD as second indication for EryDex given high unmet need and welldescribed clinical benefits of steroids in patients with DMD

#### **Well-positioned to execute**

- Cash runway through pivotal Phase 3 topline results into 2026
- Seasoned and experienced leadership team