

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

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## **Quince Therapeutics investment highlights**

> Pivotal Phase 3 clinical trial of EryDex underway in pediatric rare disease

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

Encouraging Phase 3 clinical trial results in prior study of patients with A-T

Risk-mitigated Phase 3 study conducted under Special Protocol Assessment agreement with Fast Track and Orphan Drug designations

Selected Duchenne muscular dystrophy (DMD) as second indication for EryDex

Solution State State



## No currently approved treatments for A-T patients



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

Neurological symptoms worsen until patients are wheelchair dependent, usually by age 12

S Median lifespan of approximately 25-30 years

S Currently no approved treatments for A-T

 $\bigcirc$  Similar epidemiology to Friedreich's ataxia



## Unique drug/device combination with high barriers to entry

- One-touch, automated, and point-of-care device
- Designed to deliver therapeutic in patient's own (autologous) red blood cells – distinct from complicated cell or gene therapy
- Flexible technology designed to deliver wide range of therapeutics from small and large molecules to biologics
- IP exclusivity until at least 2034 globally & 2035 in U.S. and CE mark in Europe
- More than \$100 million invested in proprietary AIDE technology



Autologous Intracellular Drug Encapsulation (AIDE) Technology



## Phase 3 lead asset EryDex encapsulates potent antiinflammatory steroid in autologous RBCs



- Autologous RBCs potentially ideal for steroid delivery
- May confer numerous benefits compared to conventional therapies, including:
  - Unique biodistribution
  - Altered pharmacokinetics and pharmacodynamics
  - Improved biocompatibility
  - Mitigating chronic toxicity and adrenal suppression
- AIDE technology designed to fundamentally alter steroid concentrations and allow for chronic administration



## Favorable EryDex safety profile compared to conventional steroid administration

Well known toxicities of conventional steroid administration		Favorable EryDex safety profile observed in clinical studies to date
Growth suppression	X	$\checkmark$
Delayed puberty	X	$\checkmark$
Immune suppression	X	$\checkmark$
Hyperglycemia	X	$\checkmark$
Excessive weight gain	X	$\checkmark$
Hirsutism	X	$\checkmark$
Acne	X	$\checkmark$

EryDex Clinical History Snapshot

- ~270 patients treated with at least one dose
- ~200 of those were patients with A-T
- Nearly 6,000 doses administered

## Attractive commercial opportunity for EryDex lead indication

# **\$1+ billion**\* estimated global

peak commercial opportunity for A-T indication alone  Estimated 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



\*\$1+ billion estimated global peak commercial opportunity and 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.

## Physicians expect broad usage of EryDex in A-T



"I would use this in as many ambulatory patients as possible. The disease has a devastating course – I would look forward to using this to try to slow down progression."

- Pediatric Neurologist

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



lote: Analysis based target product profile. Source: 2021 Third-Party Market Landscape Assessment, physician n = 14

## 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
- (> 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



## **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**



## Key clinical and corporate milestones

2024	2025	
S First patient enrolled in Phase 3 NEAT clinical trial	Ompletion 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	Prepare for potential NDA and MAA submissions in 2026, assuming positive study results	
Received Fast Track designation for A-T from FDA	Initiate DMD clinical study for second EryDex indication	
Initiation of Phase 3 NEAT open label extension	> Potential out-licensing of ex-U.S. regional territories t	
> Phase 3 ATTeST data published in The Lancet Neurology	provide runway through approval	

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## Key investment takeaways

## **O** Compelling 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