

**FINANCIAL SNAPSHOT** 

NASDAQ/TSX: AEZS

SHARE PRICE:\* \$.35

MARKET CAP:\* ~\$22.1M

SHARES OUTSTANDING: ~62.6M

\*As of September 23, 2020

Investor and

Media Relations

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Specialty biopharmaceutical company commercializing and developing therapeutics and diagnostic tests

#### Where We Are Today



First and only approved oral drug indicated for diagnosis of adult growth hormone deficiency (AGHD)







Partnership with global leader in growth hormone deficiency therapeutic market in U.S. and Canada

• Receiving royalties on sales and milestone payments

#### **Growth Opportunities**

#### **MACIMORELIN**

Market in Europe and Globally



Robust business development effort ongoing to seek additional commercialization partners

Childhood-Onset Growth Hormone Deficiency (CGHD)



Leveraging AGHD approval and compelling safety profile<sup>1</sup>

#### BUILDING A PIPELINE BEYOND DIAGNOSTICS

Macimorelin as a Therapeutic



Exploring the potential of macimorelin as a therapeutic agent in various indications

Pipeline Expansion



Evaluating opportunities to expand development pipeline

1:Macrilen [prescribing information]. Plainsboro, NJ: Novo Nordisk Inc; 2018; Macimorelin summary of product characteristics (SmPC) approved by European Commission

### Macimorelin Commercial Rights

Ongoing business development activities to secure commercialization partner in Europe and ROW

Marketed in the United States Under License and Assignment Agreement





Distribution and We know The Way
Commercialization Agreement in
Israel and the Palestine Authority



Owns Worldwide Rights
Outside of U.S. and Canada

# Market Overview Growth Hormone Deficiency



Children

Number of potential tests annually



27,000 - 28,800<sup>1</sup>



~25,0001

: Navigant study 2009, TVG study 2017, Huron Consulting study 2018





28,000 - 62,000<sup>1</sup>



14,000 -28,000<sup>1</sup>

### Leveraging Clinical Success and Compelling Safety Profile

**Expand Macimorelin Into CGHD Through Use of Pediatric Development Plan** 

CGHD Clinical Development Strategy

Dose Finding Study (P01)

Positive results announced April 2020

Provides Framework for Study P02

Efficacy Study (P02): Test Efficacy and Safety

Expected commencement date

des Framework r Study P02 2021



- Co-Development with Novo Nordisk (NN)
- NN responsible for funding 70% of the CGHD clinical trials

## **Expected Value Driving Milestones**

- ✓ Announced positive results from CGHD dose ranging study (AEZS-130-P01)
  - ✓ Distribution and commercialization agreement with MegPharm in Israel and the Palestine Authority

Planned completion of Study P02 in CGHD Earliest macimorelin CGHD approvals -FDA and EMA

December 2019

Q2 2020

Q1 2021

Q2 2022

Q3 2022

H1 2023

Inclusion of macimorelin in the AACE Growth Hormone Deficiency 2019 Guidelines Planned start of CGHD safety and efficacy study (AEZS-130-P02 - multinational, including U.S.)

Planned submission - FDA and EMA CGHD dossiers

Potential upside by pipeline activities