# ÆTERNA ZENTARIS

In Pursuit of Medical Innovations

Corporate Presentation
January 2021

# Forward Looking Statements

This document contains forward-looking statements (as defined by applicable securities legislation) made pursuant to the safe-harbor provision of the U.S. Securities Litigation Reform Act of 1995 and forward-looking information (as defined under applicable Canadian securities laws), which reflect our current expectations regarding future events. Forward-looking statements and forward-looking information may include, but are not limited to statements preceded by, followed by, or that include the words "will," "expects," "believes," "intends," "would," "could," "may," "anticipates," and similar terms that relate to future events, performance, or our results. Forward-looking statements and forward-looking information contained in this presentation are based on our opinions, estimates and assumptions in light of our experience and perception of historical trends, current conditions and expected future developments, as well as other factors that we currently believe are appropriate and reasonable in the circumstances. Despite a careful process to prepare and review the forward-looking statements and forward-looking information, there can be no assurance that the underlying opinions, estimates and assumptions will prove to be correct. Forward-looking statements involve known and unknown risks and uncertainties, including those discussed in this presentation and in our Annual Report on Form 20-F, under the caption "Key Information - Risk Factors" filed with the relevant Canadian securities regulatory authorities in lieu of an annual information form and with the U.S. Securities and Exchange Commission. Known and unknown risks and uncertainties could cause our actual results to differ materially from those in forward-looking statements and forward-looking information. Such risks and uncertainties include, among others, our ability to continue as a going concern dependent, in part, on the ability of Aeterna Zentaris Inc. to secure additional financing, our now heavy dependence on the success of Macrilen™ (macimorelin) and related out-licensing arrangements and the continued availability of funds and resources to successfully develop and commercialize the product, the ability of the Company to enter into out-licensing, development, manufacturing and marketing and distribution agreements with other pharmaceutical companies and keep such agreements in effect, the Company's ability to identify therapeutic uses for macimorelin or to in-license other product candidates, reliance on third parties for the manufacturing and commercialization of Macrilen™ (macimorelin), potential delay or termination or lack of success of our pediatric clinical trial program, potential disputes with third parties, leading to delays in or termination of the manufacturing, development, out-licensing or commercialization of our product candidates, or resulting in significant litigation or arbitration, and, more generally, uncertainties related to the regulatory process, our ability to efficiently commercialize or out-license Macrilen™ (macimorelin), the degree of market acceptance of Macrilen™ (macimorelin), our ability to obtain necessary approvals from the relevant regulatory authorities to enable us to use the desired brand names for our product, the impact of securities class action litigation or other litigation on our cash flow, results of operations and financial position, our ability to take advantage of business opportunities in the pharmaceutical industry, our ability to protect our intellectual property, the potential of liability arising from shareholder lawsuits and general changes in economic conditions. Readers of this presentation should consult our quarterly and annual filings with the Canadian and U.S. securities commissions for additional information on risks and uncertainties. Given these uncertainties and risk factors, readers are cautioned not to place undue reliance on these forward-looking statements and forward-looking information. We disclaim any obligation to update any such factors or to publicly announce any revisions to any of the forward-looking statements or forward-looking information contained herein to reflect future results, events or developments, unless required to do so by a governmental authority or applicable law.

**Certain Other Matters**

Any graphs, tables or other information demonstrating our historical performance or any other entity contained in this presentation are intended only to illustrate past performance of such entities and are not necessarily indicative of our future performance or such entities. This presentation does not constitute an offer to sell or a solicitation of an offer to buy or acquire securities of Aeterna Zentaris Inc. in any jurisdiction or an inducement to enter into investment activity, nor may it or any part of it form the basis of or be relied on in connection with any contract or commitment whatsoever. Any reference to "$" or "dollars" means United States dollars.

# Where We Are Today

## Investment Highlights

Macrilen™ 60 mg (macimorelin) for oral solution

### Macimorelin

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

FDA

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

novo nordisk®

### United States and Canada Commercial and Development Partner

Novo Nordisk is the global leader in growth hormone deficiency therapeutic market

- *Receiving royalties on sales and milestone payments*
- *Collaborating on development of macimorelin for childhood-onset growth hormone deficiency testing*

Consilient Health

### European Commercial Partner

Consilient Health is a privately owned pharma company focused on commercializing medicines in Europe and the Middle East

- *Receiving royalties on sales and milestone payments*

# Growth Opportunities

**Childhood-Onset Growth Hormone Deficiency (CGHD) Testing**

Leveraging AGHD approval and compelling safety profile[1]

- *Clinical development strategy with the goal of obtaining approval defined*
- *Preparation for pivotal study ongoing*
- *New patent filed for CGHD diagnosis*

**Macimorelin as a Therapeutic**

Working with The University of Queensland

- *Evaluating for the treatment of an undisclosed neurodegenerative disease*
- *Planning to conduct preclinical studies*
- *The University to apply for funding to conduct also investigator initiated clinical trial (IIT)*

**Pipeline Expansion**

Evaluating further 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

# 2020 Set The Stage for Future Growth

## Key Accomplishments

### Macimorelin

- Positive results in dose-range-finding pediatric study confirming dose and excellent safety profile
- Preparations to initiate pivotal Phase 3 safety and efficacy study P02
- Megapharm: distribution agreement to commercialize in Israel and Palestine Authority
- Novo Nordisk: amended commercialization and development license agreement
- Consilient Health: licensing agreement to commercialize in Europe and United Kingdom

### Corporate

- Begun key initiative to build a pipeline beyond diagnostics
- Expanded IP portfolio with additional patent applications
- Secured significant capital to fund operations through 2023
- Received €5 million up-front payment under amended agreement with Novo Nordisk
- Received €1 million up-front payment under agreement with Consilient Health
- Raised $23.5 million over the course of the year

Key 2021 Initiative

# Building A Pipeline Beyond Diagnostics

## Developing Therapeutics to Secure Long-Term Growth

# Macimorelin as a Potential Therapeutic

## Potential additional uses for Ghrelin Agonists

Semi-rational approach – (Catalent PBPK-analysis - followed for identification of:

- alternative formulations
- alternative routes of administration

Alternative, non-oral formulations preferred for therapeutic use

Experimental work on formulations ongoing

In parallel, evaluation of potential indications together with external experts

**PBPK-analysis:**
Physiologically based pharmacokinetic analysis

# Macimorelin as a Potential Therapeutic

THE UNIVERSITY OF QUEENSLAND
AUSTRALIA

Material Transfer Agreement (MTA) with University of Queensland

Exclusive rights for AEZS to negotiate a license agreement for the commercial use of the results

Initially targeting as a potential treatment for an undisclosed neurodegenerative disease

University researchers to secure grants and conduct preclinical and clinical studies

AEZS as co-investigator providing medication as well as pre-clinical and clinical know-how

# Activating Currently Owned Assets

Strategic review of current assets to identify potential program to activate and advance

- Development candidates from our previous programs may offer opportunities for re-purposing
- Various kinase inhibitors (NCEs) were already evaluated clinically or at least pre-clinically
- Ready to consider potential co-development opportunities

# Evaluating Licensing Opportunities

Successful, extensive network with universities in Europe and U.S.

Strategic collaborations with universities offer access to innovative development candidates in different indications

Focus on orphan drug indications and potential pediatric therapeutics with significant commercial potential

Assessment of in-licensing therapeutic development projects ongoing

Aim to quickly transition from pre-clinical to clinical development

# Macimorelin

## A Disruptive Oral Diagnostic Solution for GHD

# Growth Hormone is Critical to Lifelong Health

Produced by the pituitary gland
(located at the base of the brain)

## Children

**Promotes growth**

**Reduction in auxological parameters:**

- Short stature
- Low growth velocity (speed) for age
- Increased fat around the waist
- Delayed tooth development

## Adults

**Maintains normal body stature and regulates metabolism**

**No clear signs or symptoms, but recognized by:**

- Metabolic syndrome
- Osteoporosis
- Muscle wasting
- Impaired quality of life

**Increased risk of:**

- Cardiovascular (CV) issues
- Bone fractures

# Macimorelin

## Only Approved Oral Diagnostic for GHD

### Adult Growth Hormone Deficiency

- U.S. FDA approved
- European Commission approved

### Childhood-Onset Growth Hormone Deficiency

- Positive results in dose finding study announced April 2020
- Planned safety and efficacy study to commence Q1 2021

## American Association of Clinical Endocrinologists 2019 Guidelines

"Because the **macimorelin** test is simple, well tolerated with minimal side effects, and of shorter duration with only 3 to 4 blood draws compared to other GH–stimulation tests, it is anticipated that its use will increase over time." [1]

"Very promising test that is easy to conduct with high reproducibility, safety, and diagnostic accuracy comparable to the ITT and GHRH plus ARG test" [1]



1: AACE 2019 Guidelines: American Association Of Clinical Endocrinologists And American College Of Endocrinology Guidelines For Management Of Growth Hormone Deficiency In Adults And Patients Transitioning From Pediatric To Adult Care, 2019

# Leveraging Clinical Success and Compelling Safety Profile

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

**Unified Clinical Protocol Agreed with U.S. FDA and EMA**


# 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

**Q1 2021**

- Co-Development with Novo Nordisk (NN)
- Aeterna Zentaris to be the responsible Sponsor overseeing the study
- NN to fund 100% of budgeted Study P02 trial expenses up to €9 million
- Potential additional expenses to be shared

# Preparing to Initiate Pivotal Study P02

## Open-label, single dose, multicenter, multinational

- Macimorelin GHST will be performed twice (for repeatability data)
- Two standard GHSTs as controls: arginine (i.v.), clonidine (p.o.)
- Design suitable to support claim for potential of macimorelin as stand-alone test

Children and adolescents from 2 to less than 18 years of age with suspected GHD to be enrolled

2 (Years) <18

≥ 100 subjects worldwide

≥ 40 pre-pubertal and 40 pubertal subjects

≥ 25 subjects expected to be enrolled in the U.S.

# Corporate Overview

# Macimorelin Commercial Rights

## Actively seeking commercial partners in ROW

Consilient Health

**License Agreement**

- Territories: Europe and the United Kingdom
- Pricing and reimbursement milestones
- Royalties on sales
- Aeterna Zentaris controls supply chain and provides finished product according to supply agreement

novo nordisk

**License and Assignment Agreement**

- Territories: United States and Canada
- Royalties on sales
- Sales milestones
- Aeterna Zentaris controls API supply chain and provides API
- Co-development for expansion into CGHD
  - Novo Nordisk to fund 100% of budgeted Study P02 trial expenses up to €9 million
  - Potential additional expenses to be shared

ÆTERNA ZENTARIS

Owns Worldwide Rights Outside of U.S., Canada, Europe, Israel and Palestine Authority

MegaPharm We Know The Way

Distribution and Commercialization Agreement in Israel and the Palestine Authority

# Financial Snapshot

NASDAQ: AEZS / TSX: AEZS

**Cash runway to fund operations through 2023**[1]

| ~$43M | ~62.6M | ~3.2M |
| --- | --- | --- |
| Market Cap[2] | Shares Outstanding | Avg. Volume 3M[1] |



1: Based on Management's current expectations and planned development activities
2: Based on January 14, 2021 closing price of $0.68 per share

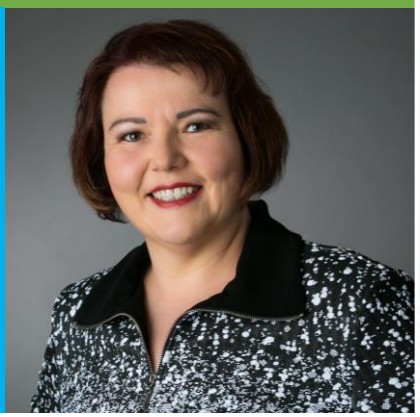
# Management

**Klaus Paulini, PhD**
*President and*
*Chief Executive Officer;*
*Managing Director,*
*Aeterna Zentaris GmbH*

**Eckhard Guenther, PhD**
*Managing Director /*
*Senior VP, Business Development*
*Aeterna Zentaris GmbH*

**Leslie Auld, CPA, MBA**
*Senior VP,*
*Chief Financial Officer*

**Nicola Ammer, MD**
*Senior VP, Clinical Development,*
*Chief Medical Officer*



# Expected Value Driving Milestones

- ✓ Successful completion of Study P01
- ✓ Amended agreement with Novo Nordisk
- ✓ European Licensing Agreement with Consilient Health Ltd.


Planned start of CGHD clinical safety and efficacy study (AEZS-130-P02 – multi-national, including U.S.)

Planned completion of P02 CGHD study according to EMA

Planned submission - FDA and EMA CGHD dossiers

Earliest macimorelin CGHD approvals – FDA and EMA

## Potential upside by additional pipeline activities

# Investment Summary

| | |
|---|---|
| FDA EUROPEAN MEDICINES AGENCY SCIENCE MEDICINES HEALTH | Macimorelin is the only approved oral product for diagnosis of adult growth hormone deficiency |
| novo nordisk® | Partnered with global leader in GHD therapeutics for U.S. and Canada<br><br>Funding 100% of budgeted Study P02 trial expenses up to €9 million |
| Consilient Health | Commercial partner seeking pricing and reimbursement approval in Europe and UK |
|  | Expanding macimorelin into CGHD, an interesting worldwide market and area of significant unmet need |
| Macimorelin | Working with The University of Queensland to explore macimorelin as a potential therapeutic for the treatment of an undisclosed neurodegenerative disease; Exclusive option to negotiate license agreement |
|  | Evaluating further opportunities to re-establish a development pipeline |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