NASDAQ: AEZS / TSX: AEZS



April 2021 Corporate Presentation

# 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 forwardlooking information (as defined under applicable Canadian securities laws), which reflect the current expectations regarding future events of Aeterna Zentaris Inc. (the "Company", "we", "us" or "our"). Forwardlooking 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. Such statements include, but are not limited to, the potential of Macrilen™ (macimorelin) to treat childhood-onset growth hormone deficiency, the size, timing and scope of our commercial and development pipeline, the potential use of Macrillen™ (macimorelin) as a therapeutic, the potential of any of our the pre-clinical products or technologies to be successfully developed, the characteristics and commercial potential of any of our products or technologies, our cash runway to fund operations and the expected timing of future key milestones, studies, agreements and approvals. 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. 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 forwardlooking information. Such risks and uncertainties include, among others, our ability to continue as a going concern is dependent, in part, on our ability to secure additional financing, our 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 Macrilen™ and our in-licensed products and technologies, the ability of the Company to enter into licensing, development, manufacturing and marketing and distribution agreements with other pharmaceutical companies, universities or others and keep such agreements in effect (including that the Company the Company may be unable to successfully negotiate a license agreement for any technology or products for which it has an option), the Company's ability to identify therapeutic uses for Macrillen™ (macimorelin) or to in-license other product candidates, the Company's reliance on third parties for the manufacturing and commercialization of Macrillen<sup>TM</sup> (macimorelin), potential delay or termination or lack of success of any of our pre-clinical or clinical programs, potential disputes with third parties leading to delays in or termination of the manufacturing, development, licensing or commercialization of our products or resulting in significant litigation or arbitration, and, more generally, uncertainties related to the regulatory process, the degree of market acceptance of Macrilen (macimorelin), the impact of securities class action litigation, shareholder lawsuits or other litigation on our cash flow, results of operations and financial position, our ability to protect our intellectual property, general changes in economic conditions and the impact of the COVID-19 pandemic on our operations, plans and prospects, including to the initiation and completion of clinical trials in a timely manner or at all.

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. The forward-looking statements and information in this presentation are made as of the date hereof and 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 that of any other entity contained in this presentation are intended only to illustrate past performance of such entities and are not necessarily indicative of future performance. This presentation does not purport to contain all of the material information with respect to the Company and is not a recommendation that any person should make an investment in the Company. Moreover, this presentation does not constitute an offer to sell or a solicitation of an offer to buy or acquire securities of the Company 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







#### Macimorelin

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





### 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

#### **European Commercial Partner**

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

Expected to receive royalties on sales and milestone payments



# Growth Opportunities

Childhood-Onset Growth Hormone Deficiency (CGHD) Testing



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

Macimorelin as a Therapeutic



Working with
The University of Queensland

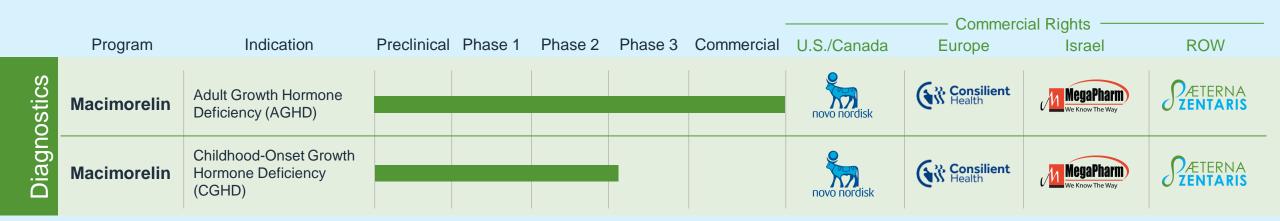
Pipeline Expansion



Advancing Therapeutic and Vaccine Programs



# Commercial and Development Diagnostic Pipeline





# Therapeutic and Vaccine Development Pipeline

	Program	Indication	Preclinical	Phase 1	Phase 2	Phase 3		
Therapeutics	AIM Biologicals	Neuromyelitis Optica Spectrum Disorder (NMOSD)					In-licensed program in January 2021	Julius-Maximilians- UNIVERSITÄT WÜRZBURG
	Macimorelin	Undisclosed Neurodegenerative Disease					Entered material transfer agreement and option to in-license in January 2021	THE UNIVERSITY OF QUEENSLAND AUSTRALIA
	Delayed Clearance Parathyroid Hormone (DC- PTH) Fusion Polypeptides	Primary Hypoparathyroidism					In-licensed program in March 2021	The University Of Sheffield.
/accine	Oral Coronavirus Vaccine Platform	Covid-19 (SARS- CoV-2)					In-licensed program in March 2021	Julius-Maximilians- UNIVERSITÄT WÜRZBURG



# Executing on Key 2021 Initiative Building A Pipeline Beyond Diagnostics

Developing Therapeutics and Vaccines to Secure Long-Term Growth



# AIM Biologicals

Targeted Immunosuppressive Therapeutics



Potential treatment option for neuromyelitis optica spectrum disorder (NMOSD)



NMOSD is an auto-antibody mediated inflammatory CNS orphan disorder with significant unmet need



Entered exclusive license and R&D agreement with Julius-Maximilians-University of Wuerzburg in January 2021



Targeted, highly specific, autoimmunity modifying proteins



Technology derived from the body's natural process that protects a fetus against the mother's immune system



Plans to conduct further preclinical research to identify and characterize lead candidate



# The Promise of Targeted, Physiological, Antigen-Specific Immunosuppression



# Immunosuppressive Drugs

Reduce disease symptoms

May cause severe side effects



#### Autoimmune Diseases are

Enhanced by autoreactive effector T cells

Ameliorated by protective effector T cells



# Antigen-specific Immunosuppression

Various antigens

may be used for selective de-sensibilization Relevant side effects may be avoided

# Macimorelin as a Potential Therapeutic



Entered Material Transfer Agreement (MTA) with University of Queensland in January 2021

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

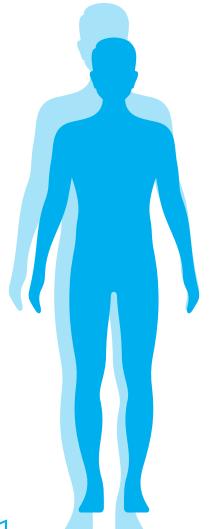


University researchers to secure grants and conduct preclinical and clinical studies

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



### Rationale



Majority of motor neuron disease (MND) patients have a moderate to marked GH deficiency

Ghrelin mimetics (agonists) like macimorelin have the potential to:



Reduce neuroinflammation

Slow metabolism

Slow weight loss



Stimulate appetite

Such effects may slow disease progression and may extend survival



# Delayed Clearance Parathyroid Hormone (DC-PTH) Fusion Polypeptides



PTH is a key regulating hormone essential for calcium homeostasis and renal phosphate clearance



Potential to be a selfadministered pen to help maintain normal serum calcium and phosphate levels



Entered exclusive license in March 2021

#### **Hypoparathyroidism**

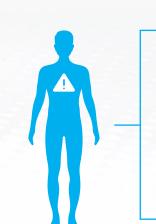
Body produces abnormally low levels of PTH

affects

Orphan indication

 $\sim$  23-37 per 100,000

causes



Renal dysfunction
Muscle cramping
Twitching
Seizures
Cardiac arrhythmias



# Oral Coronavirus Vaccine Platform



Orally active live-attenuated bacterial vaccine based on the salmonella typhi Ty21a carrier strain



Currently undergoing preclinical studies for the prevention of coronavirus diseases, including COVID-19 (SARS-CoV-2)



Entered exclusive license and research agreement with Julius-Maximilians-University of Wuerzburg in March 2021



Bundesinstitut für Impfstoffe und biomedizinische Arzneimitte Working closely with regulators on development path forward

~2°C - 8°C

Potential for temperature stable supply chain

### Multiple-Antigens

Higher likelihood for improved defense against mutated virus variants

Salmonella Typhi Ty21a carrier strain has been safely used worldwide in more than 150 million administered doses



# 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





#### **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 early Q2 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." <sup>1</sup>

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



# 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

Pivotal Study (P02): Test Efficacy and Safety

Expected commencement date





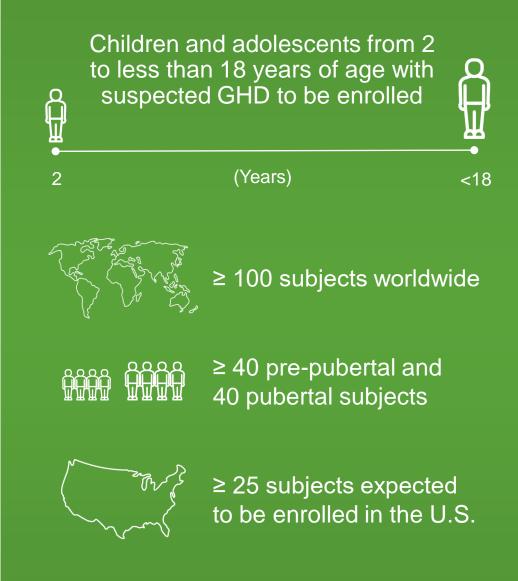
- Co-Development with Novo Nordisk (NN)
- Aeterna Zentaris to be the responsible Sponsor overseeing the study
- NN to fund 100% of budgeted Study P02 trial expenses up to €9 million
- Potential additional expenses to be shared



# 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





# Corporate Overview



# Macimorelin Commercial Rights

Actively seeking commercial partners in ROW





#### **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



#### **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



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



**Commercialization Agreement** 

in Israel and the Palestine Authority



# Financial Snapshot

NASDAQ: AEZS / TSX: AEZS

Cash runway expected to fund operations beyond 2023<sup>1</sup>

~\$133M Market Cap<sup>2</sup>

~120M Shares Outstanding<sup>3</sup> ~21M 3 month Avg. Volume<sup>4</sup>



<sup>1:</sup> Based on Management's current expectations and planned development activities

<sup>2:</sup> Based on April 5, 2021 closing price of \$1.11 per share on NASDAQ and the number of issued and outstanding AEZS shares on that date

<sup>3:</sup> Information as at February 22, 2021

<sup>4:</sup> Based on information as at April 5, 2021 for the 3 month average daily trading volume on NASDAQ

# 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 submission - FDA and EMA CGHD dossiers

2020 Q1 2021 Q2 2021 July 2022 H2 2022 H1 2023

- ✓ In-licensed NMOSD program from Julius-Maximilians-University
- ✓ In-licensed oral COVID-19 vaccine from Julius-Maximilians-University
- In-licensed DC-PTH fusion polypeptides from the University of Sheffield

Planned completion of P02 CGHD study according to EMA

Earliest macimorelin CGHD approvals – FDA and EMA

Potential upside by additional pipeline activities



# Investment Summary



Executing on key 2021 initiative to build a pipeline beyond diagnostics

- AIM Biologicals for the treatment of neuromyelitis optica spectrum disorder (NMOSD)
- Exploring macimorelin as a potential therapeutic for an undisclosed neurodegenerative disease
- DC-PTH fusion polypeptides as a potential treatment for primary hypoparathyroidism
- Oral coronavirus (COVID-19) vaccine platform





Macimorelin is the only approved oral product for diagnosis of adult growth hormone deficiency



Partnered with global leader in GHD therapeutics for U.S. and Canada

Funding 100% of budgeted Study P02 trial expenses up to €9 million



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



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