zentaris.com NASDAQ: AEZS / TSX: AEZS



Corporate Presentation October 2020

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 inlicense other product candidates, reliance on third parties for the manufacturing and commercialization of Macrillen (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 TM (macimorelin), the degree of market acceptance of Macrillen™ (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



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

Aeterna Zentaris Owns Worldwide Rights Outside of U.S. and Canada



Growth Opportunities

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 profile¹

- Clinical development strategy with the goal of obtaining approval defined
- New patent filed for CGHD diagnosis

Macimorelin as a Therapeutic



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

 New formulations and administration routes under assessment Pipeline Expansion



Evaluating opportunities to expand development pipeline



Macimorelin Commercial Rights

Ongoing business development activities to secure commercialization partner in Europe and 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 and shared costs for expansion into CGHD





Growth Hormone Deficiency



Growth Hormone is Critical to Lifelong Health



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







Growth Hormone Deficiency

Rare Endocrine System Disorder Characterized by the Inadequate Secretion of Growth Hormone

Children

Reduction in auxological parameters:

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



Adult

No single signs or symptoms, but recognized by:

- Metabolic syndrome
- Osteoporosis
- Muscle wasting
- Low physical/mental energy
- Impaired quality of life

Increased risk of:

- Cardiovascular (CV) issues
- Bone fractures



Market Overview

Growth Hormone Deficiency



Children

Number of potential tests annually



27,000-28,800¹



~25,000



Adult

Number of potential tests annually



28,000-62,000¹



14,000-28,000¹



Current GHD Diagnostic Tests

Recommended by the Endocrine Society¹

No Other FDA or EC Approved Oral Test

- Insulin tolerance test (ITT)
 - Not FDA or EC approved or regulated
- Glucagon stimulation test (GST)
 - Not FDA or EC approved or regulated
- Growth hormone releasing hormone²
 (GHRH) test with and without arginine
 - Not FDA or EC approved or regulated
 - Available in certain countries (national approvals), including the EU-5 territories³

"[ITT Test] is increasingly used less frequently in the U.S. because of safety concerns."

AACE 2019 Guidelines For Management of Growth Hormone Deficiency In Adults and Patients Transitioning From Pediatric To Adult Care



Macimorelin

A Disruptive Oral Diagnostic Solution for GHD



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

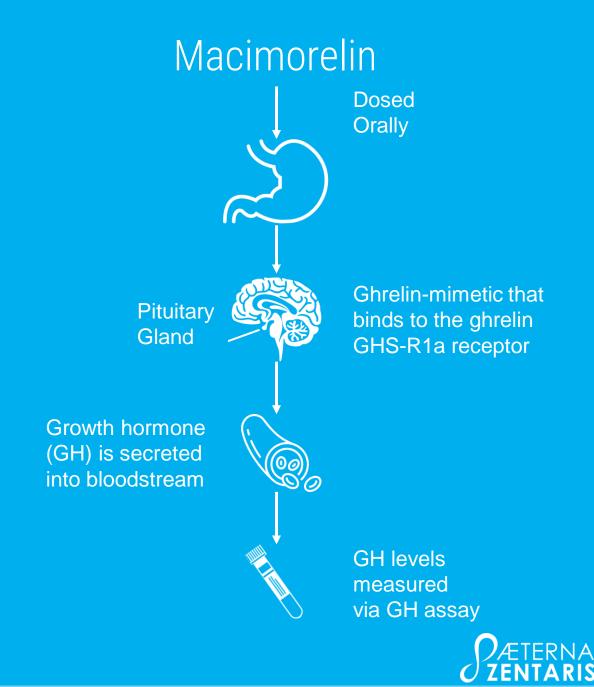
"Because the **macimorelin** test is simple, well tolerated with minimal side effects, and of shorter duration with only 3 to 4 blood draws compared to other GH–stimulation tests, it is anticipated that its use will increase over time." ¹

"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



Test Overview and Mechanism of Action Macimorelin

- Macimorelin dosing as test for AGHD:
 - 0.5 mg/kg body weight
- 1 sachet = patient up to 120 kg (265 lbs)
- The solution in mL equals the patient's body weight in kg
- 77 kg adult patient would need 77 mL

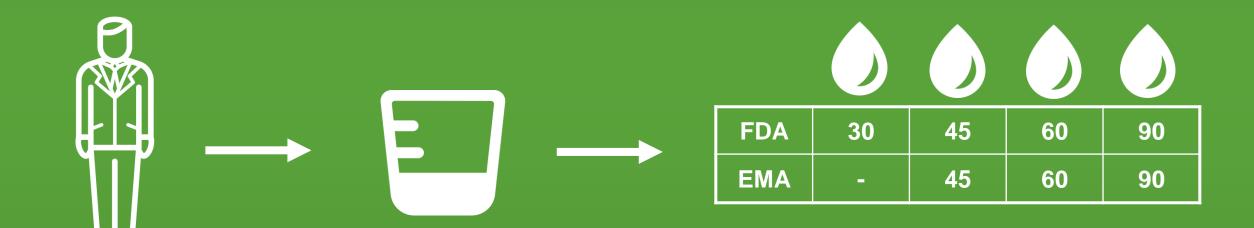


Adult Test Procedure

Macimorelin

No Physician Supervision Required

Nurse Administered



Fasted Patient (at least 8 hours)

Patient Drinks Solution
1 ml (0.5 mg/kg)
per kg body weight

Blood Draws (Minutes)



Accurately Diagnosed AGHD Compared with ITT¹

Results from an Open-Label, ITT-Controlled, 2-Way, Randomized Crossover Study

Insulin Tolerance Test (ITT)
Outcome

Positive² Negative³ **Total Subjects** Positive² 59 55 Outcome Negative³ 19 62 81 **Total Subjects** 74 66 140 **AGREEMENT** 74% 94% 84%

Positive outcome: GH release below pre-defined cut-off point (2.8 ng/mL Mac.; 5.1 ng/mL ITT) **Negative outcome:** GH release above pre-defined cut-off point

1: Garcia et al., J Clin Endocrinol Metab, 2018, 103(8), 3083-3093; 2: FDA: "...the sensitivity of the new test is estimated as the proportion of subjects with the target condition in whom the test is positive"; 3: FDA: "...the specificity of the test is estimated as the proportion of subjects without the target condition in whom the test is negative"* Source for 1 and 2: "Statistical Guidance on Reporting Results from Studies Evaluating Diagnostic Tests" - Guidance for Industry and FDA Staff as of March 13, 2007, pages 21-23

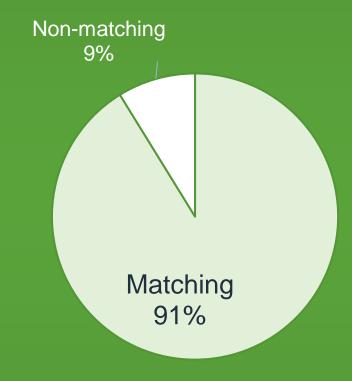
- Positive agreement higher (89%) for high-risk AGHD category
- Negative agreement 86-94%
- Compelling label with strong overall safety profile
- <1% of macimorelin tests were not evaluable vs. 17% of ITTs
- Macimorelin was highly reproducible (91%) in the same patient



Macimorelin Stimulation Test

Repeatable in 91% of Subjects¹

	AGHD Likelihood Group					
	A: High	B: Intermediate	C: Low	Total		
Number of subjects	13	12	9	34		
Subjects with match between 1st and 2nd macimorelin test	13	11	7	31		



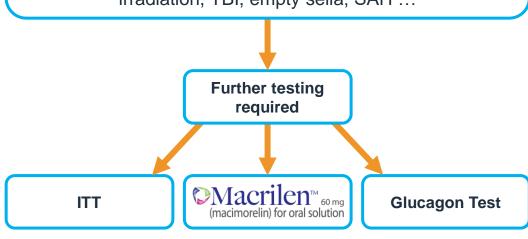


Potential to Displace Current Market as Standard Diagnosis

Current AGHD Diagnostic Process¹

Adult patients with suspicion of GHD

Low IGF-1 levels, < 3 hormone deficiencies, and/or history of hypothalamic-pituitary tumors, surgery, cranial irradiation, TBI, empty sella, SAH ...



If 1-2 stimulation tests positive



Physician Sentiment

"I think this could be the new standard of care for adult selective GH testing... too bad it's not approved in kids." U.S. Pharmacy Director

"This test could be the single exclusive test our clinic would use when GH secretion tests are needed."
U.S. KOL

"I think there is a huge potential; at the moment ITT and glucagon have great drawback. Bitter taste is a small price to pay." U.S. KOL



Significant Advantages Over Currently Available Tests

Macimorelin Is the Only Approved Oral Drug Indicated for Diagnosis of AGHD

Test	Accurate?	Safe?	Tolerability?	Simple?	Speedy?	Availability?	Cost?
ITT	Gold standard	No (in some pts)	No (in some pts)	No	No	Yes	\$
GST	Yes	Yes	No (in some pts)	Yes	No	Yes	\$
Macimorelin	Yes	Yes	Yes	Yes	Yes	Yes	\$\$\$



Childhood-Onset Growth Hormone Deficiency

Represents Significant Expansion Opportunity



Leveraging Clinical Success and Compelling Safety Profile

Expand Macimorelin Into CGHD 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





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

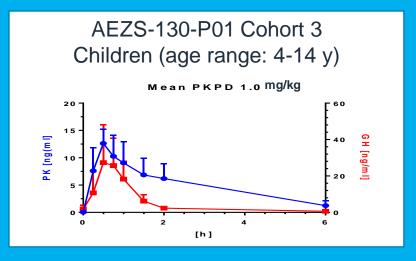


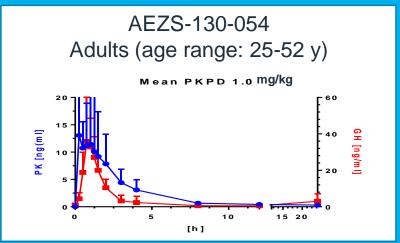
Positive Study P01

Dose Finding Pediatric Study of Macimorelin

- Study established dose for Study P02
 1mg/kg appears to lead to a strong GH stimulation
- Excellent safety and tolerability profile
 No adverse event related to macimorelin
- ~70% of AEs were related to hypoglycemia induced by the Insulin-Tolerance-Test (ITT)

PK and PD Profile Generally Comparable to Data in Adults



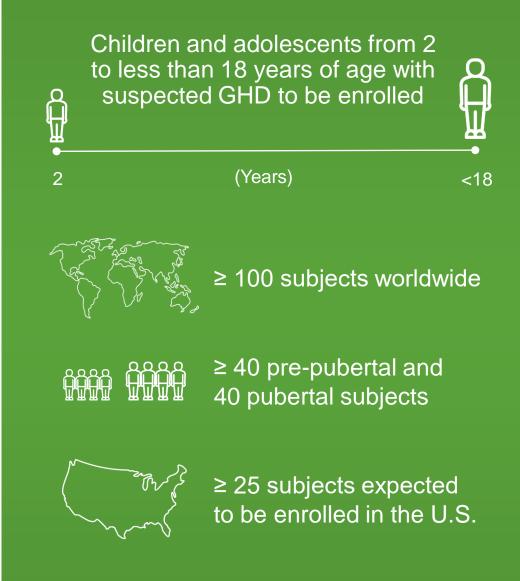




Preparing to Initiate Pivotal Study P02

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





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

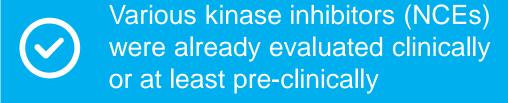


Activating Currently Owned Assets

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











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

Assessment of in-licensing of such development projects ongoing



Focus on orphan drug indications and potential pediatric use

Aim to quickly transition from pre-clinical to clinical development



Corporate Overview



Financial Snapshot

NASDAQ: AEZS / TSX: AEZS

Cash runway to fund operations through 2023¹

~\$23M Market Cap²

~62.6M Shares Outstanding ~4.65M Avg. Volume 3M¹



Management



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



Eckhard Guenther, PhD
Managing Director,
Aeterna Zentaris GmbH

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



Nicola Ammer, MD
VP Clinical Development,
Chief Medical Officer





Expected Value Driving Milestones

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

Planned completion of P02 CGHD study according to EMA

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



Investment Summary





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



Partnered with global leader in GHD, Novo Nordisk, for U.S. and Canada



Expanding macimorelin into CGHD, an interesting worldwide market and area of significant unmet need



Ongoing business development effort to secure marketing partner for macimorelin in Europe and other key markets



Evaluating opportunities to re-establish a development pipeline



NASDAQ: AEZS / TSX: AEZS



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