



Aeterna Zentaris Expands Orphan Drug Development Pipeline with Targeted Immunosuppressive Therapeutics

- Company licenses exclusive worldwide rights to develop, manufacture and commercialize targeted, highly specific immunosuppressive therapeutic proteins for the potential treatment of neuromyelitis optica spectrum disorder (“NMOSD”) from Julius-Maximilians-University of Wuerzburg, Germany

- Initial step in growth strategy to build-out pipeline of assets

- Aeterna Zentaris to develop potential therapeutic treatment option for neuromyelitis optica spectrum disorder (“NMOSD”), an orphan indication with strong unmet medical need and significant market opportunity

CHARLESTON, S.C., January 28, 2021 -- Aeterna Zentaris Inc. (NASDAQ: AEZS) (TSX: AEZS), through its wholly-owned subsidiary Aeterna Zentaris GmbH, (“Aeterna” or the “Company”), a specialty biopharmaceutical company commercializing and developing therapeutics and diagnostic tests, today announced that it has licensed the exclusive worldwide rights to develop, manufacture and commercialize targeted, highly specific, autoimmunity modifying proteins (“AIM Biologicals”) for the potential treatment of neuromyelitis optica spectrum disorder (“NMOSD”) from the Julius-Maximilians-University Wuerzburg (the “University”).

“This demonstrates Aeterna’s commitment to execute on our stated plans of expanding our pipeline to have multiple assets in research and development. The work that Dr. Valentin Bruttel and Prof. Joerg Wischhusen have conducted at the University represents what we believe to be a compelling opportunity for innovative development in the high-value indication NMOSD, an orphan indication with significant unmet need,” commented Dr. Klaus Paulini, Chief Executive Officer of Aeterna.

Prof. Joerg Wischhusen of the University added, “Based on pre-clinical data obtained by the University to date, the AIM Biologicals technology has the potential to be a breakthrough in the treatment of autoimmune-related diseases. It is based on a mechanism developed by nature to protect the fetus from the mother’s immune system without compromising immune protection against foreign antigens. This has the potential to offer a new treatment for NMOSD patients. We believe that the collaboration with Aeterna will accelerate the further development towards the clinic.”

Autoimmunity Modifying (“AIM”) Biologicals - Targeted Immunosuppressive Therapeutics

During pregnancy, the maternal immune system tolerates paternal antigens from the embryo but is still effective to protect mother and embryo from foreign antigens. Parts of the natural mechanisms responsible for this feto-maternal immune tolerance form the scientific basis for the concept of AIM Biologicals.

AIM Biologicals utilize a novel mechanism which is believed to demonstrate that peptide antigens presented on immunosuppressive MHC class I molecules can selectively and efficiently induce antigen-specific tolerance. Based on this mechanism, the targeted immunosuppressive therapeutics are being designed as optimized soluble molecules with the goal that they may be adapted to selectively induce tolerance to various autoantigens. Pre-clinical studies conducted by the University thus far indicate that tolerance induction appears to be achieved via selective elimination of antigen-specific immune effector cells and via induction of antigen-specific regulatory T cells from naïve T cells. AIM Biologicals thus have the potential to become highly specific and effective yet not personalized treatments of NMOSD.

For the treatment of NMOSD, it is believed that the AIM Biologicals will present a specific antigen derived from the water channel protein aquaporin-4 (AQP4) loaded to soluble immunoregulatory HLA-G protein to selectively induce immunological tolerance in the central nervous system.

In collaboration with the University and the University clinic, Aeterna plans to conduct further pre-clinical research to identify and characterize an AIM Biologicals based development candidate for the treatment of NMOSD, including meeting with the regulatory authorities to confirm the further pre-clinical data required as we work towards advancing such candidate into human clinical trials.

About Neuromyelitis Optica Spectrum Disorder (NMOSD)

NMOSD is an antibody mediated inflammatory central nervous system (“CNS”) disorder that affects about one per million population per year. NMOSD, also known as Devic disease, is a chronic disorder of the brain and spinal cord dominated by inflammation of the optic nerve (optic neuritis) and of the spinal cord (myelitis). Typical symptoms include visual loss, muscle spasms, paraparesis, and incontinence. If left untreated, 50% of individuals with NMOSD will be wheelchair bound and blind, and 30% will have died within five years after the first attack. The water channel protein AQP4 is widely expressed in the brain, spinal cord, and optic nerves. Auto-antibodies directed against the AQP4 channel play an important role in the pathogenesis of NMOSD.

Currently there are only three approved medications available for the treatment of NMOSD with very high annual treatment costs, and the risk of the patient contracting

serious infections. Therefore, there is a strong medical need to offer new therapeutic options to the patients.

In the U.S. and Europe there are currently approximately 10,000 to 15,000 patients living with NMOSD. Of these the AQP4 antibody seropositive patients who represent about 80% of the NMOSD population are the targeted patients for a potential therapy based on the AIM Biologicals technology.

Transaction Terms and Conditions

Under the terms of the exclusive patent license agreement entered into with the University, Aeterna obtained worldwide rights to develop, manufacture and commercialize products for the treatment of NMOSD using the AIM Biologicals technology for an up-front cash payment of €100,000 and milestone payments to be paid upon the achievement of certain development and regulatory milestones as well as royalty payments on net sales. Aeterna will be responsible for the formal preclinical and clinical development, regulatory activities, and manufacturing of the licensed products. Aeterna has also engaged the University and University clinic to conduct certain pre-development activities with respect to the AIM Biologicals program to be funded by Aeterna.

The Company intends to continue balancing risks and secure growth opportunities by re-establishing a diversified, yet focused, development pipeline to which Aeterna can best leverage its expertise and experience. Aeterna is focused on opportunistically utilizing its network with universities in Europe and the U.S. The license of the AIM Biologicals program for NMOSD demonstrates Aeterna's progress towards achieving its goal to obtain access to innovative development candidates in different indications, with a focus on rare or orphan indications with potential significant commercial opportunity.

About Aeterna Zentaris Inc.

Aeterna Zentaris Inc. is a specialty biopharmaceutical company commercializing and developing therapeutics and diagnostic tests. The Company's lead product, macimorelin, is the first and only U.S. FDA and European Commission approved oral test indicated for the diagnosis of adult growth hormone deficiency (AGHD). Macimorelin is currently marketed in the United States under the tradename Macrilen™ through a license agreement with Novo Nordisk where Aeterna receives royalties on net sales. According to a commercialization and supply agreement, MegaPharm Ltd. will seek regulatory approval and then commercialize macimorelin in Israel and the Palestinian Authority. Additionally, upon receipt of pricing and reimbursement approvals, Aeterna expects that macimorelin will be marketed in Europe and the United Kingdom through a recently established license agreement with Consilient Health Ltd. and Aeterna will receive royalties on net sales and other potential payments.

Aeterna is also leveraging the clinical success and compelling safety profile of macimorelin to develop it for the diagnosis of childhood-onset growth hormone deficiency (CGHD), an area of significant unmet need.

Aeterna is actively pursuing business development opportunities for the commercialization of macimorelin in Asia and the rest of the world, in addition to other non-strategic assets to monetize their value. For more information, please visit www.zentaris.com and connect with the Company on [Twitter](#), [LinkedIn](#) and [Facebook](#).

Forward-Looking Statements

This press release 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, which reflect our current expectations regarding future events. Forward-looking statements include those relating to the potential to obtain the necessary pre-clinical data and regulatory approvals necessary to advance any product using the AIM Biologicals technology into human clinical trials or to develop the AIM Biologicals to treat NMOSD or any other indication into an approved product, the ability of any product using the AIM Biologicals technology to compete with existing approved products (or any other products in development) for NMOSD, the ability of the Company to obtain approval of macimorelin for CGHD, the Company's ability to secure marketing partners for macimorelin in other key markets, the timing of the commencement of the CGHD Study P02, and 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", "potential" and similar terms that relate to future events, performance, or our results. Forward-looking statements involve known and unknown risks and uncertainties, including those discussed in this press release 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. Such risks and uncertainties include, among others, our ability to raise capital and obtain financing to continue our currently planned operations, our ability to continue to list our Common Shares on the NASDAQ, 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 commercialize the product, including our heavy reliance on the success of the License Agreement with Novo Nordisk, the global instability due to the global pandemic of COVID-19, and its unknown potential effect on our planned operations, including studies, our ability to enter into out-licensing, development, manufacturing, marketing and distribution agreements with other pharmaceutical companies and keep such agreements in effect, our reliance on third parties for the manufacturing and commercialization of Macrilen™ (macimorelin), 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, uncertainties related to the regulatory process, unforeseen global instability, including the instability due to the global

pandemic of the novel coronavirus, our ability to efficiently commercialize or out-license Macrilen™ (macimorelin), our reliance on the success of the pediatric clinical trial in the European Union (“E.U.”) and U.S. for 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, our ability to successfully negotiate pricing and reimbursement in key markets in the E.U. for Macrilen™ (macimorelin), the outcome of our pre-clinical and clinical development efforts of in-licensed products (including the AIM Biologicals), any evaluation of potential strategic alternatives to maximize potential future growth and shareholder value may not result in any such alternative being pursued, and even if pursued, may not result in the anticipated benefits, our ability to take advantage of business opportunities in the pharmaceutical industry, our ability to protect our intellectual property, and the potential of liability arising from shareholder lawsuits and general changes in economic conditions. Investors 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. We disclaim any obligation to update any such factors or to publicly announce any revisions to any of the forward-looking statements contained herein to reflect future results, events or developments, unless required to do so by a governmental authority or applicable law.

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