



Acadia Pharmaceuticals Announces Trofinetide New Drug Submission for Treatment of Rett Syndrome Has Been Accepted for Filing and Priority Review by Health Canada

April 22, 2024

SAN DIEGO--(BUSINESS WIRE)--Apr. 22, 2024-- Acadia Pharmaceuticals Inc. (Nasdaq: ACAD) today announced that Health Canada has accepted its New Drug Submission (NDS) for trofinetide for the treatment of Rett syndrome, a rare neurodevelopmental disorder. Health Canada has granted Priority Review for Acadia's submission.

"Rett syndrome is a profoundly debilitating and complex neurodevelopmental disorder that presents differently across patients and can lead to an array of unpredictable symptoms," said Pamela di Cenzo, Vice President, General Manager, Rare Disease, Canada at Acadia. "If granted marketing authorization, trofinetide will be the first option available to treat Rett syndrome in Canada."

Health Canada grants Priority Review for drug submissions intended for the treatment, prevention, or diagnosis of serious, life-threatening, or severely debilitating illnesses or conditions for which there is substantial evidence of clinical effectiveness that the drug addresses an unmet medical need or provides a benefit/risk profile that is improved over existing therapies.

"O.R.S.A. is pleased that Health Canada has granted Priority Review for this promising treatment which, if approved, would be a significant step forward in addressing the unmet medical needs of Canadians living with Rett syndrome," said Sabrina Millsom, President of the Ontario Rett Syndrome Association (O.R.S.A.). "Our community of patients, caregivers and supporters are excited at the prospect of having a treatment option for Rett syndrome."

The Health Canada filing is supported by results from the positive pivotal Phase 3 LAVENDER™ study evaluating the efficacy and safety of trofinetide versus placebo in 187 girls and young women with Rett syndrome. The co-primary endpoints were change from baseline in the Rett Syndrome Behaviour Questionnaire (RSBQ) total score, a caregiver assessment, and Clinical Global Impression–Improvement (CGI-I) scale score, clinician perspective, at week 12; both were statistically significant. The key secondary endpoint measuring improvements in communication was also statistically significant. Trofinetide has been approved for the treatment of Rett syndrome in adult and pediatric patients two years of age and older in the United States, and it is not currently authorized for sale in Canada for the treatment of Rett syndrome.

About Rett Syndrome

Rett syndrome is a rare genetic neurodevelopmental disorder that occurs primarily in females following a near normal development in the first two years of life.^{1,2} It is caused by mutations on the X chromosome on a gene called MECP2.³ Rett syndrome is a complex and multisystem disorder that causes profound impairment to central nervous system (CNS) function, including loss of communication skills, purposeful hand use, gait abnormalities, and stereotypic hand movements such as hand wringing/squeezing, clapping/tapping, mouthing and washing/rubbing automatisms.²

Rett syndrome occurs worldwide in approximately one of every 10,000 to 15,000 female births.⁴ In Canada, prevalence of Rett syndrome is estimated to be 600 to 900 patients.⁵ Children with Rett syndrome experience a period of developmental regression between 18-30 months of age, which is typically followed by a plateau period lasting years to decades.¹⁻³ Rett syndrome is diagnosed based on clinical evaluation, typically by about three years of age.^{2,6}

About Acadia Pharmaceuticals Inc.

Acadia is advancing breakthroughs in neuroscience to elevate life. For 30 years we have been working at the forefront of healthcare to bring vital solutions to people who need them most. We developed and commercialized in the United States the first and only FDA-approved drug to treat hallucinations and delusions associated with Parkinson's disease psychosis and the first and only FDA-approved drug for the treatment of Rett syndrome. Our clinical-stage development efforts are focused on Prader-Willi syndrome, Alzheimer's disease psychosis and multiple other programs targeting neuropsychiatric symptoms in central nervous system disorders. For more information, visit us at [Acadia.com](https://www.acadia.com) and follow us on [LinkedIn](#) and [Twitter](#).

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements include all statements other than statements of historical fact and can be identified by terms such as "intends," "may," "will," "should," "can," "could," "would," "expects," "plans," "anticipates," "believes," "estimates," "projects," "predicts," "potential" and similar expressions (including the negative thereof) intended to identify forward-looking statements. Forward-looking statements contained in this press release, include, but are not limited to, statements about: (i) the potential approval of trofinetide as the first option in Canada for the treatment of Rett Syndrome, (ii) the efficacy and safety profile of trofinetide for patients with Rett Syndrome, (iii) market acceptance in Canada, including the importance of trofinetide for the treatment of Rett Syndrome, if approved, for Rett patients or families with patients with Rett Syndrome and (iv) the ability for trofinetide to address the unmet medical needs of Canadians living with Rett syndrome. Forward-looking statements are subject to known and unknown risks, uncertainties, assumptions, and other factors that may cause our actual results, performance or achievements to differ materially and adversely from those anticipated or implied by our forward-looking statements. Such risks, uncertainties, assumptions and other factors include, but are not limited to: the ability for trofinetide to deliver efficacious and safe results to patients, if approved, our dependency on the continued successful commercialization of Daybue™ in the United States, where it is approved, our ability to obtain regulatory approval of trofinetide in Canada and other jurisdictions outside the United States, our ability to protect and enhance our intellectual property; and our ability to continue to stay in compliance with applicable laws and regulations. Given the risks and uncertainties, you should not place undue reliance on these forward-looking statements. For a discussion of these and other risks, uncertainties, assumptions, and other factors that may cause our actual results, performance or achievements to differ, please refer to our

annual report on Form 10-K for the year ended December 31, 2023, filed with the Securities and Exchange Commission on February 28, 2024, as well as our subsequent filings with the Securities and Exchange Commission from time to time. The forward-looking statements contained herein are made as of the date hereof, and we undertake no obligation to update them after this date, except as required by law.

References

- ¹ Fu et al. Consensus guidelines on managing Rett syndrome across the lifespan. *BMJ Paediatrics Open*. 2020;4:1-14.
- ² Neul JL, Kaufmann WE, Glaze DG, et al. Rett syndrome: revised diagnostic criteria and nomenclature. *Ann Neurol*. 2010;68(6):944-950.
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- ⁴ May DM, Neul JL, Satija A, et al. Real-world clinical management of individuals with Rett syndrome: a physician survey. *J of Med Econ*. 26(1), 1570–1580.
- ⁵ Acadia Pharmaceuticals Inc. Data on File. Canada prevalence of Rett syndrome. February 2024.
- ⁶ Tarquinio DC, Hou W, Neul JL, et al. Age of Diagnosis in Rett Syndrome: Patterns of Recognition Among Diagnosticians and Risk Factors for Late Diagnosis. *Pediatric Neurology*. 2015;52:585-591.

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