October 16, 2024

Acadia Pharmaceuticals Announces Health Canada Approval of DAYBUE™ (trofinetide) for the Treatment of Rett Syndrome

-- DAYBUE is the first and only therapy approved in Canada for the treatment of Rett syndrome, a rare, neurodevelopmental disorder

SAN DIEGO--(BUSINESS WIRE)-- Acadia Pharmaceuticals Inc. (Nasdaq: ACAD) today announced that Health Canada has granted marketing authorization of DAYBUE™ (trofinetide) for the treatment of Rett syndrome in adult and pediatric patients two years of age and older under the Priority Review process. The Notice of Compliance authorization of DAYBUE makes it the first and only drug approved in Canada for the treatment of Rett syndrome.

Rett syndrome is a rare, complex, neurodevelopmental disorder affecting primarily females, in which patients demonstrate significant regression following apparently normal development in the first two years of life.[1,2] In Canada, prevalence of Rett syndrome is estimated to be 600 to 900 patients.[3] Symptoms may include loss of communication skills, purposeful hand use, gait abnormalities and stereotypic hand movements such as hand wringing, clapping and rubbing.[4] Most patients living with Rett syndrome will live into adulthood and require round-the-clock care.[1,5]

“Health Canada’s authorization of DAYBUE is a significant milestone for the Rett syndrome community in Canada and another step forward in Acadia’s commitment to increase access to this therapy for patients and their families,” said Catherine Owen Adams, Acadia’s Chief Executive Officer. “We look forward to bringing DAYBUE to Canadian patients with Rett syndrome as soon as possible.”

“Rett syndrome is a debilitating and complex neurodevelopmental disorder that presents differently across patients and can lead to a range of symptoms throughout a patient’s life,” said Anita Datta, MD, FRCPC, Pediatric Neurologist and Epileptologist, Co-Director of the Rett Program at BC Children’s Hospital, and Clinical Associate Professor at the Faculty of Medicine at UBC. “Until now, treatment options have primarily focused on symptomatic management, as specific therapies for Rett syndrome have not existed.”

The Health Canada filing was supported by positive results from the pivotal Phase 3 LAVENDER™ study evaluating the efficacy and safety of trofinetide versus placebo in 187 girls and young women 5-to-20 years of age 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. RSBQ is a 45-item rating scale completed by the caregiver that assesses a range of symptoms of Rett syndrome (breathing, hand movements or stereotypies, repetitive behaviors, night-time behaviors, vocalizations, facial expressions, eye gaze, and mood). The key secondary endpoint measuring the change from baseline to Week 12 in the Communication and Symbolic Behavior Scales Developmental Profile™ Infant-Toddler Checklist – Social Composite Score (CSBS-DP-IT Social) was also statistically significant when compared to placebo.[6]

“Rett syndrome not only has a significant impact on the individuals living with this disorder, but on their families, friends and loved ones as well,” said Sabrina Millson, President of the Ontario Rett Syndrome Association (O.R.S.A.). “Today’s news is a historic milestone for the Canadian Rett syndrome community, who have eagerly awaited an approved treatment. Now that this therapy has been approved, we hope it will be made accessible through private and publicly funded drug plans as quickly as possible for all those who could benefit from it.”

In Canada, DAYBUE is indicated for the treatment of Rett syndrome in adults and pediatric patients two years of age and older and weighing at least 9 kg.[7]

For more information, in Canada visit us at CA.Acadia.com, and follow us on LinkedIn and X.

About Rett Syndrome

Rett syndrome is a rare, complex, neurodevelopmental disorder that may occur over four stages and occurs in approximately one of every 10,000 to 15,000 female births worldwide.[1,2,8] In Canada, prevalence of Rett syndrome is estimated to be 600 to 900 patients.[3] 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.[2] Rett syndrome is diagnosed based on clinical evaluation, typically by about three years of age.[4,9] Rett syndrome is a complex and multisystem disorder that causes profound impairment to central nervous system (CNS) function, loss of communication skills, purposeful hand use, gait abnormalities and stereotypic hand movements such as hand wringing, clapping and rubbing.[1,4] It is caused by mutations on the X chromosome on a gene called MECP2.[10]

About DAYBUE™ (trofinetide)

Trofinetide is a synthetic analog of the N-terminal tripeptide of insulin-like growth factor 1. The mechanism by which trofinetide exerts therapeutic effects in patients with Rett syndrome is unknown.[11]

For more information, please refer to the Product Monograph for DAYBUE in Canada located here.

About Acadia Pharmaceuticals Inc.

Acadia is advancing breakthroughs in neuroscience to elevate life. Since our founding we have been working at the forefront of healthcare to bring vital solutions to people who need them most. We developed and commercialized the first and only FDA-approved drug to treat hallucinations and delusions associated with Parkinson's disease psychosis and the first and only approved drug in the United States and Canada 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 and follow us on LinkedIn and X.

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," "could," "would," "expects," "plans," "anticipates," "believes," "estimates," "projects," "predicts," "potential," "hope" 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 efficacy and safety profile of trofinetide for patients with Rett syndrome, (ii) market acceptance in Canada, including increased accessibility and the importance of trofinetide for the treatment of Rett syndrome for Rett patients or families with patients with Rett syndrome and (iii) our estimates regarding the prevalence of patients with Rett syndrome in Canada and worldwide. 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: our dependency on the commercialization of DAYBUE in Canada and the continued successful commercialization of DAYBUE in the United States, where it is approved, our ability to obtain regulatory approval of trofinetide in jurisdictions outside the United States and Canada, our ability to protect and enhance our intellectual property, risks related to the accessibility of trofinetide to patients with Rett syndrome through private and publicly funded drug plans 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 quarterly report on Form 10-Q for the quarter ended June 30, 2024, filed with the Securities and Exchange Commission, 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

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Source: Acadia Pharmaceuticals Inc.