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DAYBU® (trofinetide) Recommended for Approval in the European Union by CHMP

-- European Commission decision expected in the coming months

-- If approved, DAYBU® would become the first treatment for neurobehavioral symptoms of Rett syndrome in the European Union

SAN DIEGO--(BUSINESS WIRE)--Acadia Pharmaceuticals Inc. (Nasdaq: ACAD) today announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has adopted a positive opinion following a re-examination procedure, recommending the granting of a marketing authorization for DAYBU® (trofinetide) for the treatment of neurobehavioral symptoms of Rett syndrome in adults and pediatric patients aged five years and older. If granted marketing authorization by the European Commission, DAYBU® would be the first therapy approved for this indication in the European Union (EU).

“The CHMP’s positive opinion for DAYBU® is an important milestone in our mission to bring this innovative therapy to the EU, where there are no therapies specifically approved for the neurobehavioral symptoms of this devastating condition," said Catherine Owen Adams, Acadia’s Chief Executive Officer. “Our commitment is to make a meaningful difference in the lives of patients, caregivers, and the wider Rett community by addressing this significant unmet need, and we are very pleased with the outcome of the re-examination process."

The CHMP’s recommendation is primarily based on results from the Phase 3 LAVENDER™ study, which demonstrated statistically significant and clinically meaningful improvements in core features of Rett syndrome, as measured by the Rett Syndrome Behaviour Questionnaire (RSBQ) and Clinical Global Impression-Improvement (CGI-I) scale. Importantly, these findings indicate that treatment with DAYBU® can address some of the most impactful aspects of Rett syndrome, which severely impact quality of life for patients and caregivers.

"For decades, families in Europe affected by Rett syndrome have had no medicine specifically approved for the neurobehavioral symptoms of this condition, despite the profound impact they have on almost every aspect of daily life,” said Pedro Rocha, President of Rett Syndrome Europe. “The CHMP’s positive opinion represents hope for thousands of European Union individuals living with this devastating condition, their families and caregivers."

Following the CHMP recommendation, the European Commission will review the opinion and is expected to issue a final decision in the coming months. If DAYBU® is approved, the marketing authorization would apply to all 27 EU member states, as well as Iceland, Liechtenstein, and Norway.

About Rett Syndrome

Rett syndrome is a rare, complex, neurodevelopmental disorder and occurs in approximately one of every 10,000 to 15,000 female births worldwide.[1-3] A child with Rett syndrome generally exhibits an early period of apparently normal development until six to 18 months, when many of their skills seem to slow down or stagnate. This is typically followed by a regression phase when the child loses acquired communication skills and purposeful hand use. The child may then experience a plateau period in which they could show mild recovery in cognitive interests, but body movements remain severely diminished. As they age, those individuals living with Rett may continue to experience a stage of motor deterioration, which can last the rest of the patient’s life.[2] Rett syndrome is typically caused by a genetic mutation on the *MECP2* gene.[4] In preclinical studies, deficiency in MeCP2 function is thought to lead to impairment in synaptic communication and brain plasticity, and the deficits in synaptic function may be associated with Rett manifestations.[4-6]

Symptoms of Rett syndrome may also include development of hand stereotypies, such as hand wringing and clapping, and gait abnormalities.[7] Most individuals living with Rett syndrome typically live into adulthood and require intense round-the-clock care.[1,8]

About DAYBU®

DAYBU® (trofinetide) is a synthetic analog of the N-terminal tripeptide of insulin-like growth factor 1.

About Acadia Pharmaceuticals

Acadia is committed to turning scientific promise into meaningful innovation that makes the difference for underserved

neurological and rare disease communities around the world. Our commercial portfolio includes the first and only FDA-approved treatments for Parkinson's disease psychosis and Rett syndrome. We are developing the next wave of therapeutic advancements with a robust and diverse pipeline that includes mid- to late-stage programs in Alzheimer's disease psychosis and Lewy body dementia psychosis, along with earlier-stage programs that address other underserved patient needs. At Acadia, we're here to be their difference. 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 "may," "will," "should," "could," "would," "intends," "expects," "plans," "anticipates," "believes," "estimates," "projects," "predicts," "potential," "guidance," "continue" 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 the expected or potential approval of trofinetide or DAYBU® by the European Commission, including the potential timing of such approval, and if approved, the potential benefits of trofinetide as a treatment of the neurobehavioral symptoms of Rett Syndrome and the importance of trofinetide to patients, caregivers and the Rett community. 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 and other factors include, but are not limited to, the inherent uncertainty regarding development of product candidates, including approval of trofinetide by the European Commission; our dependency on the continued successful commercialization of our products and our ability to maintain or increase sales of our products; our ability to obtain necessary regulatory approvals to commercialize our products and product candidates; if and when approved, market acceptance of our products 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 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 period ended March 31, 2026, filed on May 7, 2026, 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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