In 2018, Acadia entered into an exclusive licensing agreement with Neuren Pharmaceuticals Ltd. to develop and commercialize trofinetide in North America, a decision that was driven by the company mission and an appreciation that Rett syndrome is a rare disease therapeutic area of high unmet need.

Acadia leadership understood that by its nature, bringing a treatment option for such a complex, rare disease through FDA review and commercialization would not be easy. However, the development of trofinetide up until that point was encouraging to a patient community facing a devastating disorder with no FDA approved options to treat Rett syndrome.

Following positive Phase 2 results, trofinetide was Phase 3-ready as an investigational treatment for an orphan disease that would allow Acadia to expand its central nervous system (CNS) focus to include rare diseases.

Acadia’s acquisition of trofinetide and success leading the therapy through FDA approval is a historic achievement. DAYBUE™ (trofinetide) is the first FDA-approved medicine indicated for the treatment of Rett syndrome for patients two years of age and older. The approval of a therapy for Rett syndrome can help increase awareness, drive further research investment and serve as a gateway to future development for Rett syndrome.
Indication
DAYBUE™ (trofinetide) is indicated for the treatment of Rett syndrome in adults and pediatric patients 2 years of age and older.

Important Safety Information

• Warnings and Precautions

  o Diarrhea: In a 12-week study and in long-term studies, 85% of patients treated with DAYBUE experienced diarrhea. In those treated with DAYBUE, 49% either had persistent diarrhea or recurrence after resolution despite dose interruptions, reductions, or concomitant antidiarrheal therapy. Diarrhea severity was of mild or moderate severity in 96% of cases. In the 12-week study, antidiarrheal medication was used in 51% of patients treated with DAYBUE.

    Patients should stop taking laxatives before starting DAYBUE. If diarrhea occurs, patients should notify their healthcare provider, consider starting antidiarrheal treatment, and monitor hydration status and increase oral fluids, if needed. Interrupt, reduce dose, or discontinue DAYBUE if severe diarrhea occurs or if dehydration is suspected.

  o Weight Loss: In the 12-week study, 12% of patients treated with DAYBUE experienced weight loss of greater than 7% from baseline, compared to 4% of patients who received placebo. In long-term studies, 2.2% of patients discontinued treatment with DAYBUE due to weight loss. Monitor weight and interrupt, reduce dose, or discontinue DAYBUE if significant weight loss occurs.

• Adverse Reactions: The common adverse reactions (≥5% for DAYBUE-treated patients and at least 2% greater than in placebo) reported in the 12-week study were diarrhea (82% vs 20%), vomiting (29% vs 12%), fever (9% vs 4%), seizure (9% vs 6%), anxiety (8% vs 1%), decreased appetite (8% vs 2%), fatigue (8% vs 2%), and nasopharyngitis (5% vs 1%).

• Drug Interactions: Effect of DAYBUE on other Drugs

  o DAYBUE is a weak CYP3A4 inhibitor; therefore, plasma concentrations of CYP3A4 substrates may be increased if given concomitantly with DAYBUE. Closely monitor when DAYBUE is used in combination with orally administered CYP3A4 sensitive substrates for which a small change in substrate plasma concentration may lead to serious toxicities.

  o Plasma concentrations of OATP1B1 and OATP1B3 substrates may be increased if given concomitantly with DAYBUE. Avoid the concomitant use of DAYBUE with OATP1B1 and OATP1B3 substrates for which a small change in substrate plasma concentration may lead to serious toxicities.

• Use in Specific Population: Renal Impairment

  o DAYBUE is not recommended for patients with moderate or severe renal impairment.

DAYBUE is available as an oral solution (200mg/mL).

Please read the accompanying full Prescribing Information, also available at DAYBUE.com.