Neuren (NEU) – ASX Announcement **17 October 2024**

DAYBUE™ (trofinetide) receives marketing authorization in Canada

Highlights:

- **Acadia Pharmaceuticals announces Health Canada approval 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**
- **First and only approved treatment for Rett syndrome in Canada**
- **Potential sales in Canada will be added to US sales for calculation of Neuren's royalties and sales milestone payments**

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) today announced that its partner Acadia Pharmaceuticals (NASDAQ: ACAD) received Health Canada approval 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. This makes it the first and only drug approved in Canada for the treatment of Rett syndrome.

In Canada, prevalence of Rett syndrome is estimated to be 600 to 900 patients.[1] 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.

For further details please refer to Acadia's announcement https://acadia.com/media/news-releases/acadia-pharmaceuticals-announces-health-canada-approval-of-daybue-trofinetide-for-the-treatment-of-rett-syndrome/

Neuren CEO Jon Pilcher commented: "We are excited to see this first approval outside the United States, which is a significant milestone in the ongoing program to expand access to DAYBUE."

Canada is included as part of the North America region under Neuren's agreement with Acadia. Potential sales in Canada will be added to US sales for calculation of Neuren's royalties and sales milestone payments.

Tiered Royalty Rates (% of net sales)[2]	
Annual Net Sales	**Rates**
≤US$250m	10%
>US$250m, ≤US$500m	12%
>US$500m, ≤US$750m	14%
>US$750m	15%

Sales Milestones payments	
Net Sales in one calendar year	**US$m**
≥US$250m	50
≥US$500m	50
≥US$750m	100
≥US$1bn	150

[1] Acadia estimates

[2] Royalty rates payable on the portion of annual net sales that fall within the applicable range. Each sales milestone payment is payable once only.

About Neuren

Neuren is developing new drug therapies to treat multiple serious neurological disorders that emerge in early childhood and have no or limited approved treatment options. Recognising the urgent unmet need, all programs have been granted "orphan drug" designation in the United States. Orphan drug designation provides incentives to encourage development of therapies for rare and serious diseases.

DAYBUE™ (trofinetide) is approved by the US Food and Drug Administration (FDA) for the treatment of Rett syndrome in adult and pediatric patients two years of age and older. Neuren has granted an exclusive worldwide licence to Acadia Pharmaceuticals Inc. for the development and commercialisation of trofinetide.

Neuren's second drug candidate, NNZ-2591, is in Phase 2 development for multiple neurodevelopmental disorders, with positive results achieved in Phase 2 clinical trials in Phelan-McDermid syndrome, Pitt Hopkins syndrome and Angelman syndrome.

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ASX Listing Rules information

This announcement was authorized to be given to the ASX by the board of directors of Neuren Pharmaceuticals Limited, Suite 201, 697 Burke Road, Camberwell, VIC 3124

Forward-looking Statements

This announcement contains forward-looking statements that are subject to risks and uncertainties. Such statements involve known and unknown risks and important factors that may cause the actual results, performance or achievements of Neuren to be materially different from the statements in this announcement.