Research as a Service

Neuren Pharmaceuticals Ltd

On the cusp of commercialisation

Pharmaceuticals, Biotechnology & Life Sciences

9th September 2022

Neuren Pharmaceuticals Ltd (ASX:NEU) is a clinical-stage drug company focused on developing new drug therapies for debilitating neurodevelopmental disorders that emerge in early childhood. The company has two patented drugs, trofinetide and NNZ-2591, which target six neurodevelopmental disorders caused be genetic mutations and which share the common characteristic of impaired connections and signalling between brain cells. Neuren has received Orphan Drug designation from the US Food and Drug Administration (FDA) for trofinetide to treat Rett syndrome and Fragile X syndrome, and for NNZ-2591 to treat Phelan-McDermid syndrome, Angelman syndrome, Prader-Willi syndrome and Pitt Hopkins syndrome. The company also has Orphan Drug approval from the EU for all indications except Prader-Willi syndrome, which the company intends to pursue separately. The Orphan Drug designation gives Neuren marketing exclusivity after authorisation for paediatric use for 7.5 years in the US and 12 years in the EU. The company has already successfully licenced trofinetide for the North American market to Acadia Pharmaceuticals which in turn is now in the process of seeking US FDA approval for a New Drug Application for trofinetide to be used to treat Rett syndrome. Neuren has highlighted that it anticipates approval in Q1 2023 with potential revenue to flow from Acadia in this year and next. Early milestone payments could deliver up to US$83m in revenue to Neuren as well as double-digit percentage royalties.

Business model

Neuren Pharmaceuticals is developing two new drug therapies to treat multiple serious neurological disorders which emerge in early childhood. Its two drug candidates are trofinetide and NNZ-2591 which between them are targeting six neurodevelopmental disorders which currently have no existing approved therapies. The lead compound, trofinetide, achieved positive results in a Phase III clinical trial for Rett syndrome and has also completed a Phase II clinical trial in Fragile X syndrome, with both programmes achieving Fast Track designation from the US Food and Drug Administration and Orphan Drug designation in both the US and European Union. The company has licenced Acadia Pharmaceuticals Inc to develop and commercialise trofinetide in North America, retaining the rights outside the region. The company is also conducting Phase II trials of its second drug candidate, NNZ-2591, for each of Phelan-McDermid syndrome, Angelman syndrome and Pitt Hopkins syndrome, with Prader-Willi syndrome to follow.

Revenues to potentially flow from North American licence

The company is poised to earn milestone payments from Acadia Pharmaceuticals once it secures approval from the US FDA for its New Drug Application for trofinetide for the treatment of Rett syndrome in adults and paediatric patients over the age of two years. If the NDA is approved, Neuren expects to earn revenues of US$83m over the course of 2022 and 2023, which includes in 2022 a US$10m milestone payment following acceptance of the NSA and in 2023 a milestone payment of US$40m following the first commercial sale of trofinetide in the US plus US$33m as Neuren's one-third share of the market value of the US Priority Review Voucher. Ongoing, the company expects double-digit percentage royalties on net sales of trofinetide on all indications and may receive payments of up to US$350m on achievement of a series of four thresholds of total annual net sales for all indications. The company is also progressing discussions with potential licensees outside North America and undertaking its Phase II trials on its second drug NNZ-2591.

Peers are pharma companies early in commercialisation

Relevant peers, in our view, are companies at a similar stage of development and Clinuvel Pharmaeuticals (ASX:CUV) and Telix Pharmaceuticals (ASX:TLX) are two that come to mind. CUV is a little further along in terms of commercialisation, having secured US FDA marketing authorisation for its first drug, SCENESSE (afamelodotide), in April 2020. TLX secured FDA approval for its lead product Illuccix in December 2021. Both companies are revenue generating.

Share Details

ASX code	NEU
Share price (8-Sept)	$6.40
Market capitalisation	$806.2M
Shares on issue	125.97M
Net cash at 30/06/2022	$31.1M
Free float	~83.5%

Share Performance (12-months)

Upside Case

- New Drug Application (NDA) for Rett syndrome submitted to the FDA
- Successfully licenced trofinetide for the North American market
- Potential for milestone payments and royalties to paid in 2022 and 2023

Downside Case

- Phase II trials on NNZ-2591 fail
- May fail to structure licensing deals outside US
- Licensee on trofinetide fails to commercialise

Catalysts

- FDA approval of NDA for Rett syndrome
- Phase II trial results for NNZ-2591
- Commercial partnerships ex-North America

Comparable companies (Aust/NZ)

Clinuvel Pharmaceuticals (ASX:CUV), Telix Pharmaceuticals (ASX:TLX)

Board and Management

Patrick Davies	Non-Executive Chair
Jon Pilcher	Managing Director/CEO
Dr Trevor Scott	Non-Executive Director
Dianne Angus	Non-Executive Director
Dr Jenny Harry	Non-Executive Director

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FINANCIAL SERVICES GUIDE

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