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The quiet catalyst: Why open-label extension trials matter for ASX biotechs

Open-label extension trials provide long-term follow-up after a main trial and are an important part of drug development for ASX biotechs.

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- **Open-label extension trials provide long-term follow-up after a main trial is completed and are a key part of drug development**
- **DXB is undertaking an open-label extension study following its pivotal ACTION3 phase III trial of lead drug candidate DMX-200 in a rare kidney disease**
- **Actinogen has an open-label extension study underway following its phase IIb/III XanaMIA trial of Xanamem in Alzheimer's disease**

While main trial results tend to attract the big headlines for biotech companies, lower-profile open-label extension (OLE) trials can tell an equally important story.

Morgans senior healthcare analyst Scott Power told Stockhead that in an OLE, patients who complete the main trial can keep taking the drug, and everyone knows what they are receiving, with no placebo or no blinding.

"Open-label extension studies provide long-term follow-up after the main trial, giving all participants access to the active treatment," he said.

"Their primary value lies in demonstrating safety and persistence of benefit over time, rather than generating definitive efficacy data.

"Positive extension results can strengthen the overall evidence package and increase confidence among regulators, clinicians and investors."

Power noted three high-profile ASX biotechs undertaking OLE trials including **Dimerix (ASX:DXB)**, **Actinogen Medical (ASX:ACW)** and **Neurizon Therapeutics (ASX:NUZ)** .

Dimerix extends phase III trial in rare kidney disease

DXB is undertaking an OLE study following its pivotal ACTION3 phase III trial of lead drug candidate DMX-200 in focal segmental glomerulosclerosis (FSGS).

The company announced last week that the first 100 patients have completed the full two-year double-blind treatment period in ACTION3, with 93 electing to continue into an OLE study.

The OLE provides participants access to treatment with DMX-200 following completion of the double-blind phase, regardless of whether they were on placebo or DMX-200 during the trial.

DXB managing director Dr Nina Webster told Stockhead biotechs undertook OLE studies because regulators, physicians and investors all benefit from understanding a medicine's longer-term profile.

"While the pivotal clinical trial may demonstrate effectiveness over two years, OLE studies can provide important insight into durability of response, long-term safety, treatment adherence and patient willingness to remain on therapy," she said.

"For rare diseases in particular, these studies can generate valuable datasets that support future regulatory interactions, pricing/reimbursement discussions and physician confidence following approval."

Webster said DXB was particularly pleased with the level of patient participation in its OLE trial of DMX-200 in FSGS.

"What is particularly notable for our ACTION3 phase III trial is that 93% of eligible patients elected to participate in the Open Label Extension study," she said.

"While participation is entirely voluntary, this exceptionally high uptake reflects a strong level of engagement from both patients and investigators and also enables Dimerix to collect real-world treatment experience and an extensive body of long-term clinical data."

Actinogen extends Phase IIb/III Alzheimer's trial

With topline results from ACW's phase IIb/III XanaMIA trial of lead candidate Xanamem in Alzheimer's disease due in November, an OLE is also underway.

In its latest quarterly results, ACW said the OLE phase of the XanaMIA trial continued to advance strongly during the quarter.

The company reported an 87% rollover rate for participants completing the main trial and starting the OLE.

The OLE enables all current and former participants, who have completed the randomised phase of the Phase IIb/III trial, the opportunity to receive active Xanamem 10mg once daily for up to 25 months.

It is designed to provide access to active Xanamem therapy for all trial participants and gather extended safety data and observational information on key efficacy endpoints.

ACW said available data from the OLE phase would be reviewed at the same time as the XanaMIA topline final results in November.

Neurizon widens access to lead ALS drug candidate

NUZ has completed an OLE study of its lead drug candidate NUZ-001 in amyotrophic lateral sclerosis (ALS), the most common form of motor neurone disease (MND).

The 12-month OLE study involved 10 participants who continued treatment following the initial phase I MEND study, which enrolled 12 people with ALS.

NUZ reported that long-term treatment at the recommended phase II/III dose appeared safe and generally well tolerated, with no treatment-related serious adverse events.

The company said several patients were still receiving NUZ-001 under the Therapeutic Goods Administration (TGA) Special Access Scheme (SAS).

NUZ-001 has progressed to Regimen I of the HEALEY ALS Platform Trial, a phase 2/3 study and one of the largest late-stage clinical trials underway in ALS.

The multi-site HEALEY trial is being run by the Sean M. Healey & AMG Center for ALS at Mass General Brigham in the US.

HEALEY aims to accelerate development of potential new ALS therapies. It tests multiple investigational drugs simultaneously and shares infrastructure across trial sites, improving efficiencies and enabling faster results.

In the double-blinded, placebo-controlled, adaptive study, participants are randomised 3:1 to receive NUZ-001 or placebo at 78 US sites.

Participants receive 10mg/kg once daily for 36 weeks, followed by a 36-week active treatment extension.

The first participant was dosed in February 2026, with enrolment closing in July 2026 after the arm was expanded from 160 participants to 250 in May. Topline results are expected in late Q2 CY27.

NUZ has said the HEALEY trial could potentially act as a registrational trial, with positive results supporting US Food and Drug Administration (FDA) traditional approval for NUZ-001.

An expanded access program for NUZ-001 has also been earmarked, funded by the US National Institutes of Health (NIH).

The program is expected to provide up to 200 people living with ALS who are otherwise ineligible for clinical trials with access to NUZ-001 for up to 96 weeks.

The Healey & AMG Center holds the NIH grant and will run the program, with NUZ acting as regulatory sponsor and supplying NUZ-001.

NUZ said ~20 physical clinical sites participating in the HEALEY trial, along with a national virtual site, were planned for the expanded access program, providing potential access across all 50 US states. Enrolment is set to start in Q1 CY27.

The program is set to generate complementary long-term safety, biomarker, survival and clinical data for NUZ-001.

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