European Union Regulatory Guidance for ATH434 Phase 2 Clinical Trial

Highlights:

- Alterity receives positive guidance from the European Medicines Agency's Committee for Medicinal Products for Human Use on its Phase 2 clinical trial for Multiple System Atrophy.
- Concurrence with Alterity's plan to target early stage MSA patients.
- Endorsement on selection of biomarker endpoints to assess pathological hallmarks of MSA.
- Agreement that ATH434 has potential as a disease modifying treatment.

MELBOURNE, AUSTRALIA AND SAN FRANCISCO, USA – 23 June 2021. Alterity Therapeutics Limited (ASX: ATH, NASDAQ: ATHE) ("Alterity" or "the Company") has received guidance from the European Medicines Agency (EMA) regarding key aspects of the Company's Phase 2 clinical trial for investigational drug ATH434 in the treatment of Multiple System Atrophy (MSA).

Alterity is actively preparing for the launch of its Phase 2 trial expected to commence in 2H of this calendar year. The EMA is the agency of the European Union responsible for the evaluation and supervision of medicinal products. More specifically, EMA provides an important role in supporting the timely and sound development of high-quality, effective, and safe medicines, for the benefit of patients. Whilst their advice is non-binding, it has the benefit of influencing improved trial designs that are more likely to generate robust and complete data to show whether a treatment works and is safe.

Given there is no approved treatment for MSA, there is currently no regulatory precedence for defining the most suitable patient population or clinical endpoints in efficacy studies, thus requiring greater consideration in developing an optimal trial design. Accordingly, Alterity has sought diverse input from clinical experts and global regulatory authorities, and is conducting a natural history study, called BioMUSE, to identify biomarkers and clinical endpoints best suited to capture efficacy signals in the Phase 2 study.

The EMA has given its support to Alterity's intention to enroll early-stage MSA patients and to utilize biomarkers to accurately diagnose these patients prior to enrolment. Improving diagnostic accuracy and targeting early-stage patients will enable Alterity to maximize the opportunity to demonstrate the efficacy of ATH434, its potentially disease modifying therapy.

ATH434 is the first of a new generation of small molecule drug candidates designed to block the accumulation and aggregation of α-synuclein. Alpha-synuclein, when aggregated in the brain, is a pathological hallmark of Parkinsonian disorders such as MSA and is considered an important biologic target for treating these neurodegenerative diseases. ATH434 is thought to achieve its biological effect on α-synuclein by binding and redistributing excess iron in areas of pathology. The EMA recognized the potential role of iron in the pathogenesis of MSA and accordingly supported the use of biomarker endpoints to assess iron content and α-synuclein pathogenesis.

As a result of interactions with the EMA, and previously with the US Food and Drug Administration (FDA), Alterity is finalizing its Phase 2 trial design including patient selection, sample size, treatment duration, as well as primary and secondary endpoints.

In addition, the bioMUSE Natural History Study being conducted at Vanderbilt University Medical Center in the US, has enrolled more than 50% of targeted patients and continues to collect vital clinical and biomarker data to inform the Phase 2 study design.

Alterity CEO Dr David Stamler said: “MSA is a devastating disease with no cure and few effective treatment options. With the valuable advice received from the EMA, we now have a clear path forward to finalize the study design and generate data that global regulatory authorities are seeking. This is another important step toward bringing much needed disease modifying treatments to individuals with MSA.”

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Authorisation & Additional information
This announcement was authorized by David Stamler, CEO of Alterity Therapeutics Limited.

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About Alterity Therapeutics Limited and ATH434

Alterity’s lead candidate, ATH434, is the first of a new generation of small molecules designed to inhibit the aggregation of pathological proteins implicated in neurodegeneration. ATH434 has been shown to reduce abnormal accumulation of α-synuclein and tau proteins in animal models of disease by restoring normal iron balance in the brain. In this way, it has excellent potential to treat various forms of atypical Parkinsonism such as Multiple System Atrophy (MSA) and Progressive Supranuclear Palsy (PSP).

ATH434 has been granted Orphan designation for the treatment of MSA by the US FDA and the European Commission.

For further information please visit the Company’s web site at www.alteritytherapeutics.com.

Forward Looking Statements

This press release contains "forward-looking statements" within the meaning of section 27A of the Securities Act of 1933 and section 21E of the Securities Exchange Act of 1934. The Company has tried to identify such forward-looking statements by use of such words as "expects," "intends," "hopes," "anticipates," "believes," "could," "may," "evidences" and "estimates," and other similar expressions, but these words are not the exclusive means of identifying such statements.

Important factors that could cause actual results to differ materially from those indicated by such forward-looking statements are described in the sections titled “Risk Factors” in the Company’s filings with the SEC, including its most recent Annual Report on Form 20-F as well as reports on Form 6-K, including, but not limited to the following: statements relating to the Company's drug development program, including, but not limited to the initiation, progress and outcomes of clinical trials of the Company's drug development program, including, but not limited to, ATH434, and any other statements that are not historical facts. Such statements involve risks and uncertainties, including, but not limited to, those risks and uncertainties relating to the difficulties or delays in financing, development, testing, regulatory approval, production and marketing of the Company’s drug components, including, but not limited to, ATH434, uncertainties relating to the impact of the novel coronavirus (COVID-19) pandemic on the company’s business, operations and employees, the ability of the Company to procure additional future sources of financing, unexpected adverse side effects or inadequate therapeutic efficacy of the Company's drug compounds, including, but not limited to, ATH434, that could slow or prevent products coming to market, the uncertainty of patent protection for the Company's intellectual property or trade secrets, including, but not limited to, the intellectual property relating to ATH434.

Any forward-looking statement made by us in this press release is based only on information currently available to us and speaks only as of the date on which it is made. We undertake no obligation to publicly update any forward-looking statement, whether written or oral, that may be made from time to time, whether as a result of new information, future developments or otherwise.