



NodThera Announces Clinical Progress for Lead NLRP3 Inflammasome Inhibitors and Candidate Selection of Novel Brain-Penetrant Compound

-Lead candidate NT-0796 achieves positive interim results from Phase 1 study, supporting further progression for the treatment of a range of inflammatory diseases-

-Second lead candidate NT-0249 advances into first-in-human Phase 1 study to enable clinical development in peripheral chronic inflammatory disease-

-Third candidate NT-0527 is announced as a novel brain-penetrant NLRP3 inflammasome inhibitor advancing through IND-enabling studies-

LEXINGTON, MA – May 10, 2022 – [NodThera](#), a clinical-stage biotechnology company developing a new class of potent and selective oral, small molecule NLRP3 inflammasome inhibitors to treat diseases driven by chronic inflammation, today announced several key advancements across the portfolio. NodThera's lead candidate, NT-0796, demonstrated positive interim results from its Phase 1 single-ascending dose (SAD) study. Additionally, the company has commenced first-in-human dosing in the Phase 1 study of its second lead candidate, NT-0249, and announced the selection of its third pipeline candidate, NT-0527 – a brain-penetrant NLRP3 inflammasome inhibitor from a novel chemotype.

The positive interim results from the SAD portion of the Phase 1 trial with NT-0796 represent early clinical proof-of-mechanism for NT-0796 as a potent NLRP3 inflammasome inhibitor. Across all dosing cohorts, NT-0796 was safe and well tolerated and shown to be orally bioavailable with a dose-proportional pharmacokinetic (PK) profile. This portion of the study also showed a dose-dependent pharmacodynamic (PD) effect through the ability to lower IL-1 β and IL-18 levels in an *ex vivo* NLRP3-stimulation assay. These results confirm the criteria to advance NT-0796 further in development and continue the ongoing multiple-ascending dose (MAD) portion of the Phase 1 study to assess brain exposure through cerebrospinal fluid (CSF) sampling.

"NT-0796 has demonstrated robust proof of mechanism and translation from preclinical studies to humans, both validating and further de-risking the development of NT-0796 as a potentially best-in-class, oral, small molecule NLRP3 inflammasome inhibitor," said NodThera's Chief Executive Officer, Adam Keeney. "We are encouraged by these first-in-human results as we work to progress NT-0796 in inflammatory diseases impacting millions of patients, many with limited to no treatment options."

Building on successful clinical progress with NT-0796, NodThera has also initiated dosing of the first healthy volunteers in the Phase 1 trial of the company's second lead candidate, NT-0249. NT-0249 is a potent inhibitor of the NLRP3 inflammasome with favorable development characteristics supporting advancement to treat chronic inflammatory diseases of the body. The primary objective of this study will be to assess the safety and tolerability of NT-0249, with secondary objectives to assess the PK and PD (ability to lower IL-1 β and IL-18 levels) after single and multiple ascending doses.

In addition to advancing two novel candidates into clinical trials, NodThera is further expanding its diverse portfolio with the announcement of NT-0527 as the third oral small molecule NLRP3 inflammasome inhibitor from a novel chemotype to be added to its pipeline. NT-0527 is uniquely designed to inhibit the NLRP3 inflammasome in the brain, with potential to treat a broad range of neuroinflammatory diseases.



“Each of NodThera’s three portfolio candidates feature unique chemotypes that are distinct from one another, offering specific benefits that can be collectively used to cover a range of inflammatory diseases of the brain and body” shares NodThera’s Chief Scientific Officer, Alan Watt. “With the recent addition of NT-0527 as the third candidate in our growing pipeline, NodThera is now advancing novel CNS-penetrant and peripherally-restricted NLRP3 inflammasome inhibitors with differentiated chemistry unlike any other portfolio in the field.”

About NodThera

NodThera is a clinical-stage biotechnology company developing a new class of potent and selective NLRP3 inflammasome inhibitors for the treatment of diseases driven by chronic inflammation. Led by an experienced management team, NodThera is leveraging new insights into inflammasome biology and chemistry to build a portfolio of highly differentiated small molecule NLRP3 inflammasome inhibitors. The company was founded in 2016 by Epidarex Capital and financed by 5AM Ventures, Cowen Healthcare Investments, Epidarex Capital, F-Prime Capital, Novo Holdings, Sanofi Ventures and Sofinnova Partners. NodThera is headquartered in Lexington, MA, with additional locations in Cambridge, UK and Seattle, WA. Learn more at www.nodthera.com or follow us on [LinkedIn](#).

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