NapaJen Pharma Reports Positive Results from Phase 1 Clinical Trial of Novel RNA Interference (RNAi) Therapeutic

NJA-730 is an Anti-CD-40 Oligonucleotide Combined with Beta-Glucan Delivery Vehicle with Potential to Treat Acute Graft-versus-Host Disease (aGvHD)

Treatment Shown to be Safe and Well-Tolerated with No Serious Adverse Events Observed, Supporting Advancement into Phase 2 Clinical Development

BURLINGAME, CA AND TOKYO, JAPAN – October XX, 2020 – NapaJen Pharma, Inc., a biotherapeutics company leveraging its proprietary oligonucleotide delivery technology to develop novel agents, today announced positive results from the company's Phase 1 first-in-human clinical trial of NJA-730. NJA-730 is a novel immunosuppressive therapeutic that combines an anti-CD-40 oligonucleotide with the company's beta-glucan delivery technology.

Data from the Phase 1 study demonstrated NJA-730 to be safe and well tolerated in healthy subjects up to doses of 6 mg. There were no serious adverse events (AEs) observed during the study, and the majority of AEs were mild or moderate in severity and all AEs are transient. There was no relationship to dose in the incidence of AEs and adverse drug reactions. None of the observed adverse events were considered related to the liver or kidney. Researchers observed linear pharmacokinetics for NJA-730 and did not witness drug accumulation. The half-life of NJA-730 in blood was approximately 30 minutes, with the drug being rapidly eliminated from circulation. Based on these positive findings, NapaJen intends to initiate a Phase 2 clinical trial of NJA-730 for the prevention of acute graft-versus-host disease (aGvHD) in 2021.

"We are pleased to have successfully completed our first-in-human, Phase 1 clinical trial of NJA-730, demonstrating that the treatment is safe and well-tolerated at doses expected to be therapeutically active. Not only do these data support our continued advancement of NJA-730 through clinical development as a potential aGvHD therapeutic, they also provide critical clinical validation for our novel oligonucleotide delivery technology," said Kenji Arima, Ph.D., president and chief executive officer of NapaJen. "We look forward to initiating our planned Phase 2 trial of NJA-730 for prevention of aGvHD in patients receiving hematopoietic cell transplantation in both the U.S. and Australia in 2021, as well as continuing our important work on additional pipeline programs and partnering activities."

The Phase 1 trial, which was conducted in Australia, was a randomized, double-blind, placebo-controlled, single ascending dose (SAD) and multiple ascending dose (MAD) study investigating the safety, tolerability, and pharmacokinetics of NJA-730 in approximately 80 healthy adult male volunteers. The primary objective of the study was to evaluate the safety and tolerability of single and multiple ascending doses of NJA-730 administered intravenously. Key secondary objectives included an assessment of the pharmacokinetic profile of the compound. An overview of the study is available through the Australian New Zealand Clinical Trial Registry at: https://www.anzctr.org.au/Trial/Registration/TrialReview.aspx?id=375561&isReview=true.

About NJA-730

NJA-730, NapaJen's lead development program, is a novel immunosuppressive therapeutic that combines an anti-CD-40 oligonucleotide with the company's beta-glucan delivery technology. The compound is designed to leverage NapaJen's proprietary technology platform to enable selectively delivery to Dectin-1 positive dendritic cells and macrophages, allowing for the desired immunosuppressive activity in the target cells without off-target, systemic side effects.

In nonclinical mouse models, a mouse ortholog of NJA-730 was injected in a single dose prior to the bone marrow transplantation. As a result, this ortholog significantly prolonged the survival of these mice up to 45 days after the transplantation suggesting that NJA-730 was effective for prevention of aGVHD. NapaJen believes there are numerous immunoreactive conditions against which the compound may hold therapeutic promise.

About GvHD

GvHD is an immune reaction by which donor lymphocytes in the graft attack patients' tissues as heterogeneous, causing various health damages. The condition is classified as either acute GvHD, which occurs during the weeks immediately following the transplantation, or chronic GvHD, which occurs several months post-transplantation.

About NapaJen Novel Immune Cell-Targeted Delivery Technology

NapaJen's proprietary drug delivery platform is designed to overcome delivery limitations that continue to present challenges for the development of oligonucleotide-based therapeutics. The primary challenge in this area is the lack of delivery technologies of oligonucleotides. Due to the high instability, low bioavailability, and poor cellular uptake associated with oligonucleotides, efficient and selective delivery technologies are critical for the development of effective and safe oligonucleotide-based therapeutics. NapaJen's novel, proprietary delivery vehicle is built upon schizophyllan, a beta-glucan that specifically binds to Dectin-1, a cell surface receptor expressed on antigen presenting immune cells, namely dendritic cells and macrophages. By complexing oligonucleotides with schizophyllan, NapaJen's delivery platform uniquely enables the efficient and selective delivery of oligonucleotides to cells playing key roles in regulating immune responses.

About NapaJen Pharma, Inc.

NapaJen Pharma, Inc. founded in 2004, is a clinical-stage biotherapeutics company leveraging its proprietary delivery technology to develop novel oligonucleotide drugs. NapaJen has developed a proprietary platform technology in which oligonucleotide compounds are stably and selectively delivered to Dectin-1 positive immune cells using a schizophyllan/nucleotide complex. Leverag-

ing the versatility of the platform technology, NapaJen aims to create new oligonucleotide therapeutics in the area of immune-related conditions, such as autoimmune diseases, cancer, transplantation and vaccine, through collaborative work with industry and academia.

In addition to lead program NJA-730, NapaJen is also developing a Toll-like receptor 9 (TLR9) agonist as novel potent virus vaccine, as well as an agent with strong potency for immune activation. Additionally, the company continues to advance its novel technology platform, including partnering activities with designed to accelerate the development of nucleotide-based therapeutics. Technological feasibility and evaluation are currently underway with several large pharmaceutical and biotech firms in the US and Europe.

Contact Information:

NapaJen Pharma, Inc.
c/o
NapaJen Pharma Co., Ltd.
Mayumi Ohara
press@napajen.com

Tim Brons
Vida Strategic Partners (media)
646-319-8981
tbrons@vidasp.com