Denali Therapeutics Enters Agreement to Sell Rare Pediatric Disease Priority Review Voucher for $195 Million

June 18, 2026

- *Proceeds from transaction to support advancement of Denali's broad TransportVehicle™-enabled clinical portfolio for lysosomal storage disorders and neurodegenerative diseases*
- *Denali was awarded Priority Review Voucher following FDA approval of AVLAYAH™, the first FDA-approved biologic specifically designed to cross blood-brain barrier*

SOUTH SAN FRANCISCO, Calif., June 18, 2026 (GLOBE NEWSWIRE) -- Denali Therapeutics Inc. (Nasdaq: DNLI) today announced it has entered into a definitive agreement to sell its Rare Pediatric Disease Priority Review Voucher (PRV) for gross proceeds of $195 million. The U.S. Food and Drug Administration (FDA) awarded the PRV to Denali following accelerated approval of the enzyme replacement therapy AVLAYAH™ (tividenofusp alfa-eknm) for the treatment of Hunter syndrome (mucopolysaccharidosis type II; MPS II) in March 2026. AVLAYAH is the first FDA-approved medicine in an emerging class of biotherapeutics designed to cross the blood-brain barrier via transferrin receptor (TfR)-mediated transport.

"The Priority Review Voucher program is an important and effective mechanism to support the development of medicines for rare pediatric diseases. Monetizing this PRV strengthens our financial flexibility at a pivotal moment as we build on the momentum created by the FDA approval of AVLAYAH, the first FDA-approved biotherapeutic designed to reach the whole body, including the brain," said Alexander Schuth, M.D., Chief Operating and Financial Officer of Denali Therapeutics. "The proceeds will fuel the advancement and acceleration of our broad clinical pipeline, including additional Enzyme TransportVehicle programs for lysosomal storage disorders and Oligonucleotide and Antibody TransportVehicle programs targeting Alzheimer's and other neurodegenerative diseases."

Denali's clinical-stage portfolio includes DNL126 (ETV:SGSH) for Sanfilippo syndrome type A (MPS IIIA), DNL593 (PTV:PGRN) for *GRN*-related frontotemporal dementia, DNL952 (ETV:GAA) for Pompe disease and DNL628 (OTV:MAPT) for Alzheimer's disease. Denali also has multiple programs in the Investigational New Drug (IND)-enabling stage, including DNL921 (ATV:Abeta) for Alzheimer's disease, DNL111 (ETV:GCase) for Parkinson's disease and Gaucher disease, DNL622 (ETV:IDUA) for Hurler syndrome (MPS I) and DNL422 (OTV:SNCA) for Parkinson's disease.

The PRV transaction is subject to customary closing conditions, including expiration of the applicable waiting period under the Hart-Scott Rodino Antitrust Improvements Act.

About the Denali TransportVehicle ™ Platform

The blood-brain barrier (BBB) is essential in maintaining the brain's microenvironment and protecting it from harmful substances and pathogens circulating in the bloodstream. Historically, the BBB has posed significant challenges to drug development for central nervous system diseases by preventing most drugs from reaching the brain in therapeutically relevant concentrations. Denali's TransportVehicle ™ (TV) platform is a proprietary technology designed to effectively deliver large therapeutic molecules such as antibodies, enzymes and oligonucleotides throughout the whole body, including the brain, by crossing the BBB after intravenous administration. The TV platform is based on engineered Fc domains that bind to specific natural transport receptors, such as transferrin receptor and CD98 heavy chain amino acid transporter, which are expressed at the BBB and deliver the TV and its therapeutic cargo to the brain through receptor-mediated transcytosis. In animal models, antibodies and enzymes engineered with the TV platform demonstrate more than 10- to 30-fold greater brain exposure than similar antibodies and enzymes without this technology. Oligonucleotides engineered with the TV platform demonstrate more than a 1,000-fold greater brain exposure in primates than systemically delivered oligonucleotides without this technology. Improved exposure and broad distribution in the brain may increase therapeutic efficacy by enabling widespread achievement of therapeutically relevant concentrations of product candidates. The TV platform has been clinically validated, with AVLAYAH™ (tividenofusp alfa-eknm) as the first FDA-approved medicine leveraging transferrin receptor to cross the BBB.

About Denali Therapeutics

Denali Therapeutics Inc. is a biotechnology company pioneering a new class of biotherapeutics designed to cross the blood-brain barrier (BBB) using its proprietary TransportVehicle™ platform. With the first FDA-approved biologic specifically designed to cross the BBB, a clinically validated delivery platform and a growing portfolio of therapeutic candidates across all stages of development, Denali is advancing toward its goal of delivering effective medicines to transform life for people with neurodegenerative diseases, lysosomal storage disorders and other serious diseases. For more information, please visit www.denalitherapeutics.com.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995.

Forward-looking statements expressed or implied in this press release include, but are not limited to, statements regarding the timeline and likelihood of satisfying closing conditions for, and consummating the sale of, the Priority Review Voucher ("PRV"); expected use of proceeds from the sale of the PRV and the anticipated impact on Denali's cash runway; plans, timelines and expectations related to Denali's Enzyme TransportVehicle™ (ETV) franchise and its therapeutic and commercial potential; plans, timelines and expectations related to AVLAYAH™ (tividenofusp alfa-eknm); and statements by Denali's Chief Operating and Financial Officer. Actual results may differ materially from those expressed or implied by these forward-looking statements due to a variety of risks and uncertainties. These include, but are not limited to, uncertainties related to the FDA's policies and accelerated approval program; risks arising from adverse economic conditions and their impact on Denali's business and operations; the possibility of events or changes that could lead to the termination of Denali's collaboration agreements; challenges associated with Denali's transition to a commercial company; the ability of Denali and its collaborators to complete the development and, if approved, the commercialization of product candidates; difficulties in patient enrollment for ongoing and future clinical trials; whether the current ongoing trials have been powered sufficiently to demonstrate approvability to regulatory agencies; reliance on third-party manufacturers and suppliers for clinical trial materials; dependence on the successful development of Denali's blood-brain barrier platform technology and related programs; potential delays or failures in meeting expected clinical trial timelines; the risk that promising preclinical profiles may not be replicated in clinical settings; discrepancies between preclinical, early-stage or preliminary clinical results and outcomes from later-stage trials; the occurrence of significant adverse events or other undesirable side effects; the uncertainty surrounding regulatory approvals required for commercialization in the U.S., Europe or other international jurisdictions; Denali's ability to advance a pipeline of product candidates or develop commercially successful products; developments relating to Denali's competitors and its industry, including competing product candidates and therapies; Denali's ability to obtain, maintain or protect intellectual property rights related to its product candidates; the implementation and success of Denali's strategic plans for its business, product candidates and blood-brain barrier platform technology; Denali's ability to obtain additional capital to finance its operations, as needed; Denali's ability to accurately forecast future financial results in the current environment; and other risks and uncertainties, including those described in Denali's most recent Annual and Quarterly Reports on Forms 10-K and 10-Q filed with the Securities and Exchange Commission (SEC) on February 26, 2026 and May 7, 2026, respectively, and Denali's future reports to be filed with the SEC. Except for AVLAYAH, Denali's product candidates are investigational, and their safety and efficacy profiles have not yet been established. Denali does not undertake any obligation to update or revise any forward-looking statements, to conform these statements to actual results or to make changes in Denali's expectations, except as required by law.

Investor Contact:
Laura Hansen
hansen@dnli.com

Media Contact:
Erin Patton
epatton@dnli.com