Denali Therapeutics Announces Key Anticipated Milestones and Priorities for 2026 Including Commercial Launch of Tividenofusp Alfa for Hunter Syndrome

January 6, 2026

- Preparing for FDA approval and commercial launch of tividenofusp alfa, Denali's TransportVehicle™ (TV)-enabled investigational therapy for Hunter syndrome
- Expecting multiple clinical data readouts from pipeline programs including for Sanfilippo syndrome Type A (ETV:SGSH), granulin-related frontotemporal dementia (PTV:PGRN) and Parkinson's disease (LRRK2 inhibitor)
- Planning for initiation of first-in-human clinical studies with TV-enabled therapeutics for Alzheimer's disease (OTV:MAPT, ATV:Abeta) and Pompe disease (ETV:GAA)
- Continuing to strengthen leadership in transferrin receptor (TfR)-enabled and blood-brain barrier crossing therapeutics with continued advancement of enzyme, oligonucleotide and antibody programs

SOUTH SAN FRANCISCO, Calif., Jan. 06, 2026 (GLOBE NEWSWIRE) -- Denali Therapeutics Inc. (NASDAQ: DNLI), today announced key anticipated milestones and priorities for 2026 across its portfolio of investigational therapies for neurodegenerative diseases, lysosomal storage disorders and other serious diseases. Chief Executive Officer Ryan Watts, Ph.D., will highlight these priorities during a corporate presentation at the 44th Annual J.P. Morgan Healthcare Conference on Tuesday, January 13, at 1:30 p.m. PDT.

"2026 is a defining year for Denali as we prepare to deliver our first TV-enabled medicine to patients," said Dr. Watts. "We are on the cusp of launching tividenofusp alfa, which we believe will establish a new standard of care for people living with Hunter syndrome and mark the first commercial validation of our platform. In addition, we expect multiple clinical data readouts across our portfolio and plan to advance two TV-enabled programs into clinical studies for Alzheimer's disease and a program for Pompe disease. As pioneers in TfR-enabled therapeutics, we are committed to advancing a new generation of transformative medicines with the potential to enhance and enable delivery of biotherapeutics throughout the whole body, including the brain."

2026 Outlook

Expected progress and key milestones across Denali's portfolio of TV-enabled and small molecule programs in 2026 are summarized below.

CLINICAL PROGRAMS

Tividenofusp alfa (DNL310, ETV:IDS) for Hunter syndrome (MPS II)

Denali is preparing for commercial launch in anticipation of a regulatory decision on the Biologics License Application (BLA) for tividenofusp alfa under the U.S. Food and Drug Administration (FDA) accelerated approval pathway with a Prescription Drug User Fee Act (PDUFA) target action date of April 5, 2026. Results from the open-label Phase 1/2 clinical trial of tividenofusp alfa were published in the January 1, 2026 issue of The New England Journal of Medicine. The ongoing global Phase 2/3 COMPASS study is expected to generate confirmatory evidence and support global regulatory submissions; enrollment in Cohort A (neuronopathic participants) was completed in December 2025.

DNL126 (ETV:SGSH) for Sanfilippo syndrome Type A (MPS IIIA)

Denali will present initial clinical data from the fully enrolled, ongoing Phase 1/2 study of DNL126 at the 2026 WORLD*Symposium*™ (February 3-6, 2026). The Phase 1/2 study is designed to support an accelerated approval path in Sanfilippo syndrome Type A. Planning for a global Phase 3 confirmatory study is ongoing.

TAK-594/DNL593 (PTV:PGRN) for *GRN*-related frontotemporal dementia (FTD-*GRN*)

The Phase 1/2 study of TAK-594/DNL593 is ongoing with screening closed in Cohort B. Initial FTD-*GRN* patient data are expected in 2026. The program is being developed in collaboration with Takeda.

DNL628 (OTV:MAPT) for Alzheimer's disease

DNL628 is enabled by Denali's Oligonucleotide TransportVehicle™ (OTV) and is designed to cross the blood-brain barrier and reduce the tau protein by targeting the *MAPT* gene that encodes for tau. Denali today announced that the Clinical Trial Application (CTA) for the Phase 1b study of DNL628 has been approved and study start-up activities are underway.

DNL952 (ETV:GAA) for Pompe disease

DNL952 is enabled by Denali's Enzyme TransportVehicle [TM] (ETV) and designed to enhance delivery of the missing enzyme, GAA, into muscle tissues and across the blood-brain barrier into the brain. Denali today announced that the U.S. FDA has lifted the clinical hold on the Investigational New Drug (IND) application for DNL952, and Denali will proceed with the Phase 1 study.

BIIB122/DNL151 (small molecule LRRK2 inhibitor) for Parkinson's disease

The global Phase 2b LUMA study of BIIB122 completed enrollment of participants with early-stage Parkinson's disease in 2025, with a clinical readout expected in 2026. Denali's Phase 2a BEACON study in LRRK2-associated Parkinson's disease remains ongoing. The LRRK2 program is being developed in collaboration with Biogen.

SAR443122/DNL758 (eclitasertib; small molecule RIPK1 inhibitor) for ulcerative colitis

The Phase 2 study of eclitasertib in participants with moderate to severe ulcerative colitis is expected to have results in the first half of 2026. The program is being developed by Sanofi.

IND-ENABLING STAGE PROGRAMS

The following programs are in IND-enabling stage: DNL921 (ATV:Abeta) for Alzheimer's disease, DNL111 (ETV:GCase) for Parkinson's disease and Gaucher disease, DNL622 (ETV:IDUA) for MPS I, and DNL422 (OTV:SNCA) for Parkinson's disease.

PARTNERSHIPS

Denali has active collaborations with Biogen for BIIB122/DNL151 in Parkinson's disease and with Takeda for TAK-594/DNL593 in FTD-*GRN*, both with 50/50 U.S. commercial rights. Denali also stands to receive royalty payments for SAR443122/DNL758, which is licensed to Sanofi and in development for ulcerative colitis.

FINANCIAL OUTLOOK

As of September 30, 2025, Denali had approximately $872.9 million in cash, cash equivalents and marketable securities. In December 2025, Denali completed an equity financing with gross proceeds of approximately $200 million and announced a royalty funding agreement with Royalty Pharma based on future net sales of tividenofusp alfa with proceeds up to $275 million.

KEY ANTICIPATED 2026 MILESTONES

Program	Indication	Expected Milestone	Timing
Tividenofusp alfa (ETV:IDS)	MPS II (Hunter syndrome)	US Accelerated Approval	1H
DNL126 (ETV:SGSH)	MPS IIIA (Sanfilippo syndrome type A)	Phase 1/2 data	1H
DNL628 (OTV:MAPT)	Alzheimer's disease	Phase 1b study initiation	1H
DNL952 (ETV:GAA)	Pompe disease	Phase 1 study initiation	1H
DNL151 / BIIB122	Parkinson's disease	Phase 2b LUMA data	1H
DNL921 (ATV:Abeta)	Alzheimer's disease	Phase 1/1b study initiation	1H
DNL126 (ETV:SGSH)	MPS IIIA (Sanfilippo syndrome type A)	Phase 3 study initiation	2H
DNL593 (PTV:PGRN)	FTD-*GRN*	Phase 1/2 data	2H

Webcast details for Denali's presentation at the 44th Annual J.P. Morgan Healthcare Conference

A live and archived webcast of the Denali presentation during the J.P. Morgan Conference on Tuesday, January 13, 2026, at 1:30 p.m. PDT will be available on the Events page under the Investor section of the Denali website at https://investors.denalitherapeutics.com/events.

About Denali Therapeutics

Denali Therapeutics Inc. is a biotechnology company pioneering a new class of biotherapeutics designed to cross the blood-brain barrier using its proprietary TransportVehicle™ platform. With 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 the lives of people living with neurodegenerative, lysosomal storage and other serious diseases. For more information, please visit www.denalitherapeutics.com.

Cautionary Note Regarding 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 future results of operations and financial position of Denali Therapeutics Inc. ("Denali" or the "Company"); Denali's business strategy and business plans, including expected key milestones for Denali's therapeutic portfolio in 2026 and beyond and Denali's ability to execute on its commercial strategies; plans, timelines, expectations related to Denali's TransportVehicle™ (TV) platform and its therapeutic and commercial potential; plans, timelines, and expectations relating to tividenofusp alfa (DNL310), including the timing, likelihood, and scope of regulatory approvals and commercial launch, the establishment of tividenofusp alfa as standard of care in MPS II, the enrollment of the Phase 2/3 COMPASS study, and the likelihood of the Phase 2/3 COMPASS data to support confirmatory evidence for global regulatory submissions and approval; plans, timelines, and expectations related to DNL126, including the timing and availability of data from the Phase 1/2 study to support an accelerated approval path in MPS IIIA and the timing to initiate a Phase 3 study; plans and expectations regarding DNL593, including the ongoing Phase 1/2 study and the timing for the Phase 1/2 study data; plans, timelines, and expectations related to DNL628 (OTV:MAPT), including the Phase 1b study timing; plans, timelines, and expectations related to DNL952 (ETV:GAA), including the Phase 1 study timing; plans, timelines, and expectations related to DNL151, including the timing of availability of clinical data from the ongoing Phase 2b LUMA study and the ongoing Phase 2a BEACON study; plans, timelines, and expectations related to DNL758, including the timing for results of the Phase 2 study; plans, timelines, and expectations related to DNL921, including the timing of the Phase 1/1b study initiation; expectations regarding Denali's leadership in developing TfR-enabled and BBB-crossing therapeutics; expectations regarding Denali's preclinical studies and the timing and likelihood of advancement of additional programs to clinical studies; Denali's third-party collaborations, commercial rights, and potential royalties; Denali's financial outlook, cash position, and potential cash proceeds based on a royalty funding agreement; and statements made by Denali's Chief Executive Officer. Actual results are subject to risks and uncertainties and may differ materially from those indicated by these forward-looking statements as a result of these risks and uncertainties, including but not limited to: uncertainties related to the FDA's policies and accelerated approval program, including risks that the PDUFA action date may be extended and the FDA may not approve tividenofusp alfa; the possibility of events or changes that could lead to the termination of Denali's collaboration agreements; Denali's dependence on successful development and commercialization of its BBB platform technology and TV-enabled product candidates; Denali's ability to initiate and enroll patients in its current and future clinical trials; Denali's ability to conduct or complete clinical trials on expected timelines; Denali's reliance on third parties for the manufacture and supply of its product candidates for clinical trials and commercial products; the potential for clinical trial results to differ from preclinical, early clinical, preliminary or expected results; the risk of significant adverse events, toxicities, or other undesirable side effects; the risk that results from early clinical biomarker studies will not translate to clinical benefit in late clinical studies; the risk that product candidates may not receive regulatory approval necessary to be commercialized; 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; and other risks and uncertainties. In light of these risks, uncertainties, and assumptions, the forward-looking statements in this press release are inherently uncertain and may not occur, and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements. Accordingly, you should not rely upon forward-looking statements as predictions of future events. Denali's product candidates are investigational, and their safety and efficacy profiles have not yet been established. No Denali product candidates have been approved by any health authority for any use. Information regarding additional risks and uncertainties may be found in Denali's Annual and Quarterly Reports filed on Forms 10-K and 10-Q filed with the Securities and Exchange Commission (SEC) on February 27, 2025, and November 6, 2025, respectively, and Denali's future reports to be filed with the SEC. 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.

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