Denali Therapeutics Reports First Quarter 2026 Financial Results and Business Highlights

May 7, 2026

- *FDA approved AVLAYAH™ (tividenofusp alfa-eknm) for treatment of Hunter syndrome (MPS II) and as first medicine to leverage transferrin receptor to cross blood-brain barrier*
- *AVLAYAH launched in U.S. with strong momentum, vibrant community engagement, and first patients treated in commercial setting in April*
- *Broad clinical pipeline progressing for lysosomal storage and neurodegenerative diseases, including first patient dosed with Oligonucleotide TransportVehicle™ (OTV)-enabled DNL628 (OTV:MAPT) targeting tau for Alzheimer's disease*
- *Advancing DNL593 (PTV:PGRN) in Phase 1/2 study for GRN-related frontotemporal dementia after regaining full rights, with data expected by end of 2026*

SOUTH SAN FRANCISCO, Calif., May 07, 2026 (GLOBE NEWSWIRE) -- Denali Therapeutics Inc. (Nasdaq: DNLI) today reported financial results for the first quarter ended March 31, 2026, and provided business highlights, including the recent U.S. Food and Drug Administration (FDA) approval of AVLAYAH™ (tividenofusp alfa-eknm).

“The FDA approval of AVLAYAH is a major milestone for Denali, for the Hunter syndrome community, and for the field of biotherapeutics enabled to cross the blood-brain barrier. We are thrilled by the strong engagement with the community, seamless execution by our commercial team, and achievement of our first patient dosed in less than one month from approval,” said Ryan Watts, Ph.D., Chief Executive Officer of Denali Therapeutics. “AVLAYAH provides validation for our TransportVehicle™ (TV) platform enabling our broad clinical portfolio for lysosomal storage and neurodegenerative diseases. We are excited about progress achieved across the portfolio, including dosing of the first patients with our Oligonucleotide TV-enabled investigational therapy DNL628 (OTV:MAPT) targeting tau for Alzheimer’s disease and advancing DNL593 (PTV:PGRN) for FTD- *GRN* after regaining full rights."

First Quarter 2026 and Recent Program Updates

COMMERCIAL PRODUCT

AVLAYAH (tividenofusp alfa-eknm) for Hunter syndrome (mucopolysaccharidosis type II [MPS II])

On March 25, 2026, Denali announced AVLAYAH (tividenofusp alfa-eknm) received accelerated approval for the treatment of neurologic manifestations of Hunter syndrome (MPS II) when initiated in presymptomatic or symptomatic pediatric patients weighing at least 5 kg prior to advanced neurologic impairment. Continued approval for this indication may be contingent upon verification of clinical benefit in a confirmatory trial. The U.S. commercial launch of AVLAYAH is underway and the first patients have received therapy. All key operational launch components are in place, including availability of commercial product through an established distribution channel and fully operational patient support hub. The major health systems and key national and regional payers have been engaged. The ongoing global Phase 2/3 COMPASS study is designed to generate confirmatory evidence and support global regulatory submissions for AVLAYAH.

CLINICAL PROGRAMS

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

DNL126 is an investigational, intravenously administered, Enzyme TransportVehicle™ (ETV)-enabled N-sulfoglucosamine sulfohydrolase (SGSH) replacement therapy designed to deliver SGSH into the brain and body, with the goal of addressing the behavioral, cognitive and physical manifestations of Sanfilippo syndrome type A. The Phase 1/2 trial of DNL126 is ongoing, and start-up activities are underway for a global Phase 3 confirmatory study. Denali expects a Biologics License Application (BLA) submission and potential accelerated approval for DNL126 for Sanfilippo syndrome type A in 2027.

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

Denali is conducting a Phase 1/2 study of DNL593, an investigational, intravenously administered progranulin replacement therapy utilizing Denali’s Protein TransportVehicle™ (PTV) to deliver progranulin across the blood-brain barrier (BBB) and into the brain for individuals with FTD-*GRN*. Enrollment in the study is complete with a total of 40 participants with FTD-*GRN*, and results are expected by the end of 2026.

DNL628 (OTV:MAPT) for Alzheimer's disease

In March 2026, the first patient was dosed in the Phase 1b study of DNL628, which is an investigational therapy for Alzheimer's disease and enabled by Denali's Oligonucleotide TransportVehicle™ (OTV). DNL628 is designed to cross the BBB and reduce the tau protein by targeting the *MAPT* gene that encodes for tau. Denali expects data from this study in 1H 2027.

DNL952 (ETV:GAA) for Pompe disease

DNL952 is enabled by Denali's ETV and designed to enhance delivery of the missing enzyme, GAA, into muscle tissues and across the BBB into the brain. Phase 1 study start-up activities are underway.

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

A clinical data readout of the global Phase 2b LUMA study of BIIB122 for early-stage Parkinson's disease is expected in mid-2026. Denali's Phase 2a BEACON study in individuals with Parkinson's disease who are confirmed by genetic testing to be carriers of a pathogenic LRRK2 variant is ongoing. The LRRK2 program is being developed in collaboration with Biogen.

IND-ENABLING STAGE PROGRAMS

Denali has multiple additional programs in the 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 MPS I; and DNL422 (OTV:SNCA) for Parkinson's disease. Denali is on track to submit a regulatory filing for DNL921 in the first half of 2026 to begin clinical development of this TV-enabled anti-amyloid program for Alzheimer's disease.

Corporate Updates

As previously announced in connection with the approval of AVLAYAH, the FDA granted Denali Therapeutics a Rare Pediatric Disease Priority Review Voucher (PRV). This voucher may be used to obtain priority review for a future marketing application and can be transferred to another sponsor.

On March 27, 2026, Denali received $200 million in gross proceeds in connection with the closing of the transactions under a synthetic royalty funding agreement signed in December 2025 with Royalty Pharma Investments 2023 ICAV.

On April 3, 2026, Denali announced it received notification from Takeda of its decision to terminate the collaboration agreement between the two companies to co-develop and co-commercialize DNL593. Takeda's decision was driven by strategic considerations and was not related to efficacy or safety data. Denali continues to advance DNL593 in the ongoing Phase 1/2 study in patients with FTD-*GRN* and expects results by the end of 2026 as described above.

Participation in Upcoming Investor Conferences

- Bank of America Healthcare Conference 2026, May 12-14 (Las Vegas)
- Jefferies Global Healthcare Conference, June 2-4 (New York City)
- Goldman Sachs 47th Annual Global Healthcare Conference, June 8-10 (Miami)
- BTIG Virtual Biotechnology Conference, July 28-29

First Quarter 2026 Financial Results

Net loss was $128.4 million for the quarter ended March 31, 2026, compared to net loss of $133.0 million for the quarter ended March 31, 2025.

Total research and development expenses were $103.8 million for the quarter ended March 31, 2026, compared to $116.2 million for the quarter ended March 31, 2025. The decrease of approximately $12.4 million was primarily attributable to the timing of manufacturing of AVLAYAH commercial supply in the first quarter of 2025, as well as lower external expenses related to small molecule programs.

General and administrative expenses were $33.5 million for the quarter ended March 31, 2026, compared to $29.4 million for the quarter ended March 31, 2025. The increase of $4.1 million was primarily driven by higher personnel-related costs due to increased headcount in the first quarter of 2026, reflecting headcount additions made throughout 2025 to support post-launch activities for AVLAYAH.

Cash, cash equivalents and marketable securities were approximately $1.05 billion as of March 31, 2026.

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 expectations for Denali's TransportVehicle™ (TV) platform, including the Enzyme TransportVehicle™ (ETV) franchise, and its therapeutic and commercial potential; plans, timelines, and expectations relating to the commercial launch of AVLAYAH™ (tividenofusp alfa-eknm) and related activities; expectations related to the ongoing Phase 2/3 COMPASS study of tividenofusp alfa, including the timing and availability of data and its ability to generate confirmatory evidence and support global regulatory submissions; plans, timelines and expectations related to DNL126, including the ongoing Phase 1/2 study, the planned Phase 3 confirmatory study, the planned BLA submission, and the likelihood and timing of accelerated approval; plans, timelines and expectations related to DNL593, including the ongoing Phase 1/2 study, the timing and availability of data, and Denali's ability to independently advance the program; plans, timelines and expectations related to DNL628, including the ongoing Phase 1b study and the timing and availability of data; plans, timelines and expectations related to DNL952 and the planned Phase 1 study; plans, timelines and expectations related to DNL151, including the ongoing Phase 2a BEACON study, and the timing and availability of data from the Phase 2b LUMA study; plans, timelines and expectations related to DNL921, including the expected timing of a regulatory filing and initiation of clinical development; plans, timelines, and expectations for IND-enabling stage programs; plans and expectations regarding Denali's Rare Pediatric Disease Priority Review Voucher; expectations regarding the Royalty Pharma funding agreement, including royalty payment obligations and milestones; plans regarding participation in upcoming investor conferences; and statements by Denali's Chief Executive 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; discrepancies between preclinical, early-stage or preliminary clinical results and outcomes from later-stage trials; the risk that interim or topline clinical results may not be predictive of final study results or longer-term outcomes; 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 competing product candidates; 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 Report on Form 10-K filed with the Securities and Exchange Commission (SEC) on February 26, 2026, 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.

Denali Therapeutics Inc.
Condensed Consolidated Statements of Operations
(Unaudited)
(In thousands, except share and per share amounts)

	Three Months Ended March 31,	
	2026	2025
Operating expenses:		
Research and development	$ 103,846	$ 116,227
General and administration	33,511	29,353
Total operating expenses	137,357	145,580
Loss from operations	(137,357)	(145,580)
Interest and other income, net	8,910	12,610
Net loss	$ (128,447)	$ (132,970)
Net loss per share, basic and diluted	$ (0.69)	$ (0.78)
Weighted average number of shares outstanding, basic and diluted	186,636,978	171,222,030

Denali Therapeutics Inc.
Condensed Consolidated Balance Sheets
(Unaudited)
(In thousands)

	March 31, 2026	December 31, 2025
Assets		
Current assets:		
Cash and cash equivalents	$ 387,626	$ 205,326
Short-term marketable securities	600,058	662,553
Prepaid expenses and other current assets	35,068	32,779
Total current assets	1,022,752	900,658
Long-term marketable securities	63,785	98,322
Property and equipment, net	51,728	52,402
Finance lease right-of-use asset	47,616	48,531
Operating lease right-of-use asset	17,922	19,002
Intangible asset, net	36,000	—
Other non-current assets	26,220	25,939
Total assets	$ 1,266,023	$ 1,144,854
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 40,380	$ 505
Accrued expenses and other current liabilities	69,776	76,745
Total current liabilities	110,156	98,351
Operating lease liability, less current portion	24,680	27,210
Finance lease liability, less current portion	5,508	5,532
Liability related to the revenue participation right agreement	199,581	—
Total liabilities	339,925	131,093
Total stockholders' equity	926,098	1,013,761
Total liabilities and stockholders’ equity	$ 1,266,023	$ 1,144,854

Investor Contact:
Laura Hansen, Ph.D.
hansen@dnli.com

Media Contact:
Erin Patton
epatton@dnli.com

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