Denali Therapeutics Reports Third Quarter 2025 Financial Results and Business Highlights

November 6, 2025

- Tividenofusp alfa BLA review process for accelerated approval for MPS II continues with productive engagement with the FDA; commercial launch preparations on track
- DNL126 Phase 1/2 study enrollment completed, supporting an accelerated approval path in MPS IIIA
- Two new regulatory applications submitted to initiate clinical studies with DNL628 (OTV:MAPT) for Alzheimer's disease and DNL952 (ETV:GAA) for Pompe disease
- Tim Van Hauwermeiren, CEO of argenx, to join Denali's Board of Directors
- Carole Ho, M.D., Chief Medical Officer, departing company; Peter Chin, M.D., assuming role of Acting CMO and Head of Development
- Denali to host Investor Day on December 4, 2025

SOUTH SAN FRANCISCO, Calif., Nov. 06, 2025 (GLOBE NEWSWIRE) -- Denali Therapeutics Inc. (Nasdaq: DNLI) today reported financial results for the third quarter ended September 30, 2025, and provided business highlights.

"Momentum is building across Denali as we prepare for the anticipated launch of tividenofusp alfa with an experienced and focused commercial team in place," said Ryan Watts, Ph.D., Chief Executive Officer of Denali Therapeutics. "We are also excited to have submitted regulatory applications to initiate clinical studies with two additional programs representing new opportunities to expand the TransportVehicle ™ platform to Alzheimer's disease and Pompe disease. Our robust pipeline continues to lead the way in the emerging class of transferrin receptor (TfR)-enabled medicines designed to deliver the power of biotherapeutics throughout the body, including the brain."

Third Quarter 2025 and Recent Program Updates

CLINICAL PROGRAMS

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

In October, Denali announced that the FDA extended its review timeline of the Biologics License Application (BLA) seeking accelerated approval of tividenofusp alfa for the treatment of mucopolysaccharidosis type II (MPS II), also known as Hunter syndrome. The Prescription Drug User Fee Act (PDUFA) target date was extended from January 5, 2026, to April 5, 2026. The extension follows Denali's submission of updated clinical pharmacology information in response to an information request from the FDA as part of the standard review process and is not related to efficacy, safety or biomarkers. The FDA classified the submission as a Major Amendment (MA) to the BLA, which, per FDA regulations, extends the review by three months. No additional data were requested by the FDA in the MA letter. Denali believes that the updated information submitted in the amendment does not affect the clinical pharmacology or benefit-risk conclusions of the BLA. Denali continues to have productive engagement with the FDA on the review process while preparing for commercial launch.

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

In September, Denali completed enrollment in the ongoing Phase 1/2 study of DNL126 to support an accelerated approval path in MPS IIIA, also known as Sanfilippo syndrome type A. Previously announced data demonstrated a significant reduction in cerebrospinal fluid (CSF) heparan sulfate (HS) from baseline, including normalization, and a safety profile that supports continued development. A global Phase 3 confirmatory study is being planned. Phase 1/2 data will be presented in a platform presentation at the 2026 WORLD*Symposium* ™.

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

Denali and Takeda continue their collaboration to develop DNL593, an investigational therapeutic designed to deliver progranulin across the blood-brain barrier for the treatment of granulin (GRN) mutation-associated frontotemporal dementia (FTD-GRN). A Phase 1/2 study is ongoing.

DNL628 (OTV:MAPT) for the treatment of Alzheimer's disease

In October, the company submitted a Clinical Trial Application (CTA) for DNL628 (OTV:MAPT) to initiate clinical studies in Alzheimer's disease, marking a significant milestone in advancing the Oligonucleotide TransportVehicle ™ (OTV) platform.

DNL952 (ETV:GAA) for the treatment of Pompe disease

In October, Denali submitted an Investigational New Drug (IND) application for DNL952 (ETV:GAA) to begin clinical studies in Pompe disease, expanding the reach of the Enzyme TransportVehicle ™ (ETV) platform into muscle disease.

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

Denali and Biogen continue co-development of BIIB122. The Phase 2b LUMA study completed enrollment earlier this year with a data readout expected in 2026, while Denali's Phase 2a BEACON study in LRRK2-associated Parkinson's disease remains ongoing.

IND-ENABLING STAGE PROGRAMS

Denali expects to continue expanding its TV-enabled pipeline across enzyme, antibody, and oligonucleotide franchises, bringing forward one to two new programs annually. The next most advanced programs include: DNL921 (ATV:Abeta) for Alzheimer's disease; DNL111 (ETV:GCase) for Parkinson's/Gaucher disease; DNL622 (ETV:IDUA) for MPS I; and DNL422 (OTV:SNCA) for Parkinson's disease.

CORPORATE UPDATES

Today, in a separate press release, Denali announced that Tim Van Hauwermeiren has been appointed to its Board of Directors. Mr. Van Hauwermeiren is co-founder and Chief Executive Officer of argenx. Carole Ho, M.D., who has served as Denali's Chief Medical Officer and Head of Development since 2015, will be departing to join Eli Lilly and Company as Executive Vice President, and President of Lilly Neuroscience. Peter Chin, M.D., is assuming the role of Acting Chief Medical Officer and Head of Development at Denali. Dr. Chin is a neurologist and joined Denali in 2019, most recently serving as Senior Vice President of the ETV Franchise and Late-Stage Clinical Development.

Participation in Upcoming Investor Conferences

- Stifel 2025 Healthcare Conference, November 11 - 13 (New York City)
- Jefferies Global Healthcare Conference, November 17 - 20 (London)

Denali's 2025 Investor Day on December 4, 2025, in New York City

Denali's leadership team will host an in-person and virtual Investor Day on December 4, 2025, in New York City to provide an update on the company's progress and strategic priorities, including its transition to a fully integrated organization and preparations for the planned launch of tividenofusp alfa for Hunter syndrome (MPS II). The discussion will also highlight continued advancement of Denali's TransportVehicle platform, pipeline execution across multiple programs, and the company's near- and long-term strategy to drive sustainable growth and create shareholder value through the development of transformative treatments for people living with serious diseases. The event is scheduled to begin at 8:30 a.m. EST and will continue until approximately 11:30 a.m. EST. In-person attendance is intended for institutional investors and financial analysts. Denali is also offering a live webcast of the event, which will be accessible from the Events page of the Investor section on Denali's corporate website: www.denalitherapeutics.com.

Third Quarter 2025 Financial Results

Net loss was $126.9 million for the quarter ended September 30, 2025, compared to a net loss of $107.2 million for the quarter ended September 30, 2024.

Total research and development expenses were $102.0 million for the quarter ended September 30, 2025, compared to $98.2 million for the quarter ended September 30, 2024. The increase of approximately $3.8 million was attributable to increases of $7.8 million and $6.4 million in other research and development expenses and personnel-related expenses, respectively, both driven by the commencement of operations at Denali's large molecule manufacturing facility in Salt Lake City, Utah. These increases were partially offset by a decrease of $10.2 million in external expenses for small molecule programs.

General and administrative expenses were $35.5 million for the quarter ended September 30, 2025, compared to $24.9 million for the quarter ended September 30, 2024. The increase of $10.6 million was primarily driven by preparatory activities for a potential commercial launch for tividenofusp alfa.

Cash, cash equivalents, and marketable securities were approximately $872.9 million as of September 30, 2025.

About Denali Therapeutics

Denali Therapeutics is a biopharmaceutical company developing a broad portfolio of product candidates engineered to cross the blood-brain barrier (BBB) for the treatment of neurodegenerative diseases and lysosomal storage diseases. Denali pursues new treatments by rigorously assessing genetically validated targets, engineering delivery across the BBB, and guiding development through biomarkers that demonstrate target and pathway engagement. Denali is based in South San Francisco. For additional 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 expectations for Denali's TV platform and its therapeutics and commercial potential; statements made by Denali's Chief Executive

Officer; plans, timelines, and expectations relating to DNL310, including the PDUFA target action date and plans for commercial launch; plans, timelines, and expectations related to DNL126, including the global confirmatory Phase 3 study; plans regarding DNL593 and the ongoing Phase 1/2 study and the timing and availability of initial patient data; plans, timelines, and expectations regarding DNL628 and the initiation of clinical studies; plans, timelines, and expectations regarding DNL952 and the initiation of clinical studies; plans, timelines, and expectations regarding DNL151, including with respect to the ongoing Phase 2a BEACON study and the timing and likelihood of readout of the Phase 2b LUMA study; plans and expectations for Denali's preclinical programs, including the timing of advancement to clinical studies; plans and timelines regarding corporate changes; Denali's participation in upcoming investor conferences; expectations for Denali's 2025 Investor Day, including with respect to the planned content and availability of updates; Denali's future operating expenses and anticipated cash runway; and statements by Denali's Chief Executive Officer. All drugs currently being developed by Denali are investigational and have not received regulatory approval for any indication. 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, risks related to: the impact of adverse economic conditions, tariffs, and inflation on Denali's business and operations; the occurrence of any event, change, or other circumstance that could give rise to the termination of Denali's agreements with Sanofi, Takeda, Biogen, or other collaborators; Denali's transition to a late-stage clinical drug development company; Denali's and its collaborators' ability to complete the development and, if approved, commercialization of its product candidates; Denali's and its collaborators' ability to enroll patients in its ongoing and future clinical trials; Denali's reliance on third parties for the manufacture and supply of its product candidates for clinical trials; Denali's dependence on successful development of its blood-brain barrier platform technology and its programs and product candidates; Denali's and its collaborators' ability to conduct or complete clinical trials on expected timelines; the risk that preclinical profiles of Denali's product candidates may not translate in clinical trials; the potential for clinical trials to differ from preclinical, early clinical, preliminary or expected results; the risk of significant adverse events, toxicities, or other undesirable side effects; the uncertainty that product candidates will receive regulatory approval necessary to be commercialized; Denali's ability to continue to create a pipeline of product candidates or commercialize 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; implementation 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 and hedge against financial risk in the current environment; and other risks and uncertainties, including those described in Denali's most recent Annual Report and Quarterly Reports on Forms 10-K and 10-Q filed with the Securities and Exchange Commission (SEC) on February 27, 2025 and November 6, 2025, 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.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Operating expenses:				
Research and development	$ 101,950	$ 98,238	$ 320,873	$ 296,653
General and administrative	35,484	24,949	97,104	75,379
Total operating expenses	137,434	123,187	417,977	372,032
Gain from divestiture of small molecule programs	—	—	—	14,537
Loss from operations	(137,434)	(123,187)	(417,977)	(357,495)
Interest and other income, net	10,532	15,995	33,986	49,475
Net loss	$ (126,902)	$ (107,192)	$ (383,991)	$ (308,020)
Net loss per share, basic and diluted	$ (0.74)	$ (0.63)	$ (2.24)	$ (1.89)
Weighted average number of shares outstanding, basic and diluted	172,421,492	169,456,988	171,702,183	162,589,325

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

	September 30, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 90,963	$ 174,960
Short-term marketable securities	757,241	657,371
Prepaid expenses and other current assets	34,394	32,105
Total current assets	882,598	864,436
Long-term marketable securities	24,703	359,373
Property and equipment, net	53,732	55,236
Finance lease right-of-use asset	49,447	47,533
Operating lease right-of-use asset	20,035	22,861
Other non-current assets	25,106	24,741
Total assets	$ 1,055,621	$ 1,374,180
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 6,745	$ 11,137
Accrued compensation	18,358	24,728
Accrued clinical and other research & development costs	23,355	22,822
Accrued manufacturing costs	5,002	12,779
Operating lease liability, current	9,164	8,308
Deferred research and development funding liability, current	22,580	14,129
Other accrued costs and current liabilities	4,942	8,305
Total current liabilities	90,146	102,208
Operating lease liability, less current portion	29,686	36,673
Finance lease liability, less current portion	5,554	5,615
Deferred research funding and development liability, less current portion	4,038	—
Total liabilities	129,424	144,496
Total stockholders' equity	926,197	1,229,684
Total liabilities and stockholders’ equity	$ 1,055,621	$ 1,374,180

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Source: Denali Therapeutics Inc.