



Design Therapeutics Reports Third Quarter 2023 Financial Results and Plans for a Comprehensive Portfolio Update in Early 2024

November 13, 2023

Seasoned Biotech Executive Pratik Shah Appointed as Chief Executive Officer to Lead Company Turnaround

Current Cash and Securities of ~\$290M Support Extended Five-Year Operating Runway Through 2028

Revised Corporate Strategy with Realignment Towards Long-Term Growth to be Presented in Early 2024; Additional Details to Follow

CARLSBAD, Calif., Nov. 13, 2023 (GLOBE NEWSWIRE) -- Design Therapeutics, Inc. (Nasdaq: DSGN), a biotechnology company developing treatments for serious degenerative genetic diseases, today reported third quarter 2023 financial results. A strategic realignment was initiated to prioritize long-term growth, focus capital resources on program spend and implement cost savings. As a result, Design now expects its cash, cash equivalents and marketable securities to fund its planned operating expenses through at least the next five years. Additionally, Design announced plans to provide an update on its corporate strategy and priorities, and outline anticipated milestones across its business and clinical- and research-stage pipeline of novel GeneTAC™ small molecules in early 2024.

"When we started Design just five years ago, we were looking to address the problem of genetic diseases differently by working *alongside* a person's genome. Our solution, GeneTAC™ small molecules, are thoughtfully designed to dial up or down the expression of specific genes, addressing the root cause of disease *without* permanently modifying their genome. We believe this technology has the potential to transform the treatment landscape for many patients suffering from devastating degenerative diseases," said Pratik Shah, Ph.D., chairperson and chief executive officer of Design Therapeutics. "The recently reported data from our observational biomarker study and multiple-ascending dose Phase 1 trial in patients with Friedreich ataxia (FA) provided us with important proof-of-concept for our GeneTAC™ platform, with the first human in history with FA experiencing a restoration of mRNA to carrier levels without the need for an irreversible genetic modification. Additionally, the initial clinical results provided important learnings on the behavior and tolerability of our lead GeneTAC™ molecule, DT-216, in patients, leading to our efforts to further optimize the formulation of DT-216 containing excipients with improved injection site tolerability, to enable the long-term treatment of patients with FA."

Dr. Shah continued, "We believe that GeneTAC™ molecules can be transformative therapies for patient populations with urgent unmet medical needs, including FA, Fuchs endothelial corneal dystrophy and myotonic dystrophy type-1. To maximize our future potential, we are undertaking a close review of our business to build on the progress already made and determine the optimal paths forward and development timelines for each of our programs. Already, we have sharpened our focus to prioritize our long-term growth, allowing us to strategically deploy our capital for pipeline investment that extends our operating plans through at least the next five years. We look forward to sharing more on our near- and long-term strategic priorities as part of a comprehensive update in early 2024. This is an important time for Design, and it's an honor to lead this incredible team as we work to translate the proof-of-concept established for our platform into meaningful treatments for patients and the treating communities."

Third Quarter 2023 Financial Results

- **R&D Expenses:** Research and development (R&D) expenses were \$13.3 million for the quarter ended September 30, 2023.
- **G&A Expenses:** General and administrative (G&A) expenses were \$5.6 million for the quarter ended September 30, 2023.
- **Net Loss:** Net loss was \$15.8 million for the quarter ended September 30, 2023.
- **Cash Position and Operating Runway:** Cash, cash equivalents and marketable securities were \$290.9 million as of September 30, 2023. As a result of a strategic realignment to prioritize long-term growth, focus capital resources on program spend and implement cost savings, Design now expects its cash, cash equivalents and marketable securities as of September 30, 2023, to fund its planned operating expenses through at least the next five years.

About Design Therapeutics

Design Therapeutics is a biotechnology company developing a new class of therapies based on its platform of GeneTAC™ gene targeted chimera small molecules. The company's GeneTAC™ molecules are designed to either dial up or dial down the expression of a specific disease-causing gene to address the underlying cause of disease. In addition to its lead GeneTAC™ small molecule, DT-216, in development for patients with Friedreich ataxia, the company is advancing programs in Fuchs endothelial corneal dystrophy and myotonic dystrophy type-1. Discovery efforts for multiple other serious degenerative disorders caused by nucleotide repeat expansions are also underway, including for fragile X syndrome, spinocerebellar ataxias, Huntington disease, spinobulbar muscular atrophy, and C9orf72-amyotrophic lateral sclerosis/frontotemporal dementia. For more information, please visit designtx.com.

Forward-Looking Statements

Statements in this press release that are not purely historical in nature are "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. These statements include, but are not limited to projections from early-stage programs, nonclinical data and early-stage clinical data; the ability of an improved formulation of DT-216 to improve injection site tolerability to enable the long-term treatment of patients with FA; Design's ability to advance the GeneTAC™ platform; the potential of GeneTAC™ molecules to be transformative therapies for patient populations with

urgent unmet medical needs, including FA, Fuchs endothelial corneal dystrophy and myotonic dystrophy type-1; Design's estimated financial runway and the sufficiency of its resources to support its planned operations; and the capabilities and potential advantages of Design's pipeline of GeneTAC™ molecules. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. Words such as "believes," "designed to," "anticipates," "aims," "plans to," "expects," "estimate," "intends," "will," "potential" and similar expressions are intended to identify forward-looking statements. These forward-looking statements are based upon Design's current expectations and involve assumptions that may never materialize or may prove to be incorrect. Actual results and the timing of events could differ materially from those anticipated in such forward-looking statements as a result of various risks and uncertainties, which include, without limitation, risks associated with the acceptance of INDs by the FDA for the conduct of planned clinical trials of our product candidates and our proposed design of future clinical trials; risks associated with designing and implementing investigational drug product formulation improvements; risks associated with conducting a clinical trial and patient enrollment, which is affected by many factors, and any difficulties or delays encountered with such clinical trial or patient enrollment may delay or otherwise adversely affect Design's clinical development plans; the process of discovering and developing therapies that are safe and effective for use as human therapeutics and operating as a development stage company; the risk that undesirable side effects or other properties could cause Design or regulatory authorities to suspend or discontinue clinical trials which could delay or prevent Design's product candidates' development or regulatory approval; Design's ability to develop, initiate or complete nonclinical studies and clinical trials for its product candidates; the risk that promising early research or clinical trials do not demonstrate safety and/or efficacy in later nonclinical studies or clinical trials; changes in Design's plans to develop its product candidates; uncertainties associated with performing clinical trials, regulatory filings and applications; risks associated with reliance on third parties to successfully conduct clinical trials and nonclinical studies; Design's reliance on key third parties, including contract manufacturers and contract research organizations; Design's ability to raise any additional funding it will need to continue to pursue its business and product development plans; regulatory developments in the United States and foreign countries; Design's ability to obtain and maintain intellectual property protection for its product candidates; Design's ability to recruit and retain key scientific or management personnel; competition in the industry in which Design operates, which may result in others discovering, developing or commercializing competitive products before or more successfully than Design; and market conditions. For a more detailed discussion of these and other factors, please refer to Design's filings with the Securities and Exchange Commission ("SEC"), including under the "Risk Factors" heading of Design's Quarterly Report on Form 10-Q for the quarter ended June 30, 2023, as filed with the SEC on August 14, 2023, and under the "Risk Factors" heading of Design's Quarterly Report on Form 10-Q for the quarter ended September 30, 2023, being filed with the SEC later today. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement and Design undertakes no obligation to revise or update this press release to reflect events or circumstances after the date hereof, except as required by law.

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**DESIGN THERAPEUTICS, INC.
CONDENSED STATEMENTS OF OPERATIONS**

(in thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
	(unaudited)			
Operating expenses:				
Research and development	\$ 13,257	\$ 14,304	\$ 46,051	\$ 34,358
General and administrative	5,565	4,888	17,018	13,843
Total operating expenses	<u>18,822</u>	<u>19,192</u>	<u>63,069</u>	<u>48,201</u>
Loss from operations	(18,822)	(19,192)	(63,069)	(48,201)
Other income, net	3,033	1,488	8,049	2,233
Net loss	<u>\$ (15,789)</u>	<u>\$ (17,704)</u>	<u>\$ (55,020)</u>	<u>\$ (45,968)</u>
Net loss per share, basic and diluted	<u>\$ (0.28)</u>	<u>\$ (0.32)</u>	<u>\$ (0.98)</u>	<u>\$ (0.83)</u>
Weighted-average shares of common stock outstanding, basic and diluted	<u>55,988,691</u>	<u>55,782,329</u>	<u>55,948,867</u>	<u>55,654,490</u>

**DESIGN THERAPEUTICS, INC.
CONDENSED BALANCE SHEETS**

(in thousands)

	September 30, 2023	December 31, 2022
	(unaudited)	
Assets		
Current assets:		
Cash, cash equivalents and investment securities	\$ 290,940	\$ 330,387
Prepaid expenses and other current assets	3,603	4,732
Total current assets	294,543	335,119
Property and equipment, net	1,778	1,947
Right-of-use asset, related party	3,111	3,612
Other assets	437	459
Total assets	<u>\$ 299,869</u>	<u>\$ 341,137</u>
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	\$ 2,207	\$ 3,025
Accrued expenses and other current liabilities	10,183	7,751
Total current liabilities	12,390	10,776
Operating lease liability, net, related party	2,522	3,051
Total liabilities	14,912	13,827
Total stockholders' equity	284,957	327,310
Total liabilities and stockholders' equity	<u>\$ 299,869</u>	<u>\$ 341,137</u>