Homology Medicines Reports Fourth Quarter and Full Year 2022 Financial Results and Recent Highlights

March 9, 2023 9:05 PM EST

- On Track to Provide Initial Clinical Data from Gene Editing Trial for PKU Mid-Year with First Participant Dosed and Others in Screening -

- Initial Clinical Data from Gene Therapy Trial for Hunter Syndrome Anticipated in Second Half of 2023; Trial Recruiting in the U.S. and Canada -

- Anticipated Cash Runway Into Fourth Quarter 2024 Enables Execution Against Key Milestones -

BEDFORD, Mass., March 09, 2023 (GLOBE NEWSWIRE) -- Homology Medicines, Inc. (Nasdaq: FIXX), a genetic medicines company, announced today financial results for the fourth quarter and full year ended December 31, 2022, and highlighted recent accomplishments.

“We entered 2023 with strong momentum across our gene editing and gene therapy clinical trials for PKU and Hunter syndrome, and we anticipate initial data read-outs from both programs this year,” said Albert Seymour, Ph.D., President and Chief Executive Officer of Homology Medicines. “Dosing of the first patient in the pheEDIT trial for PKU marked a key milestone for Homology, and the forthcoming data will represent the first gene editing data in PKU patients. We believe the continued interest in the juMPStart trial for Hunter syndrome underscores the impact a one-time gene therapy with potential to address both the peripheral and CNS disease manifestations could have for patients and their loved ones.”

Continued Dr. Seymour, “We recently shared a series of data that support these programs, including preclinical work on the targeted immunosuppression regimen being utilized in both pheEDIT and juMPStart. We also unveiled details of our optimized MLD gene therapy candidate, demonstrating its ability to cross the blood-brain-barrier in the disease model with enzyme activity expected to lead to *in vivo* efficacy. The third arm of our platform, GTx-mAb, continues to advance as we move HMI-104 for PNH through IND-enabling studies. We are well-positioned to execute against key milestones with anticipated funding into the fourth quarter of 2024.”

Fourth Quarter 2022 and Recent Accomplishments

- Dosed first participant in the Phase 1 pheEDIT clinical trial evaluating *in vivo* nuclease-free gene editing candidate HMI-103 in adults with phenylketonuria (PKU); additional participants are in screening. Homology continues to expect initial clinical data from the trial mid-year 2023.
    - Shared preclinical data that showed murine surrogate of HMI-103 was ten times more potent than non-integrating gene therapy vector HMI-102 in the murine model of PKU; HMI-103 is designed with a unique mechanism of action (MOA) to maximize PAH enzyme through both genome integration and episomal expression, and it has the potential to treat adults and pediatric patients.
- Building on physician and patient interest, anticipate initial clinical data from the HMI-203 juMPStart gene therapy trial for Hunter syndrome in the second half of 2023.
- Presented data that support the targeted immunosuppressive regimen in Homology’s clinical trials. In non-human primates (NHPs), the combination of a T-cell inhibitor and steroid was most effective in reducing the immune response to AAVHSC and improving gene expression.
- Presented preclinical data with HMI-204, Homology’s optimized, *in vivo* gene therapy candidate for metachromatic leukodystrophy (MLD). A single I.V. dose in the murine model of MLD led to robust expression in the central nervous system (CNS), including sustained levels of enzyme activity reaching levels of normal human adults and predicted to lead to efficacy *in vivo*. Homology continues to seek a partner for the optimized product candidate, which is ready to enter IND-enabling studies.
- Progressed HMI-104, a C5 monoclonal antibody (mAb) development candidate for paroxysmal nocturnal hemoglobinuria (PNH), through IND-enabling studies. HMI-104 is the first candidate that utilizes the Company’s GTx-mAb platform and is focused on using the liver to express a C5 mAb with a one-time dose. Homology believes its GTx-mAb platform has the

potential to address larger market indications.
- Announced today the promotion of Julie Jordan, M.D., to Chief Medical Officer.

Fourth Quarter 2022 and Full Year Financial Results

- Net loss for the quarter ended December 31, 2022 was $(34.3) million or $(0.60) per share, compared to a net loss of $(33.6) million or $(0.59) per share for the same period in 2021. Net loss for the year ended December 31, 2022 was $(5.0) million or $(0.09) per share, compared to a net loss of $(95.8) million or $(1.73) per share for the same period in 2021. The decrease in net loss was primarily due to a gain of $131.2 million realized in connection with the Company's sale of its manufacturing business to Oxford Biomedica in order to establish Oxford Biomedica Solutions ("OXB Solutions"), an AAV Innovation and Manufacturing Business, in the first quarter of 2022, partially offset by lower collaboration revenues in 2022.
- Collaboration revenues for the three and twelve months ended December 31, 2022 were $0.8 million and $3.2 million, respectively, as compared to $0.8 million and $34.0 million for the comparable periods in 2021. Collaboration revenues in 2022 consisted of revenue recognized under the Company's stock purchase agreement with Pfizer compared with collaboration revenues in 2021, which were primarily the result of concluding the Company's collaboration with Novartis.
- Total operating expenses for the three and twelve months ended December 31, 2022 were $35.3 million and $136.5 million, respectively, as compared to $34.4 million and $129.9 million for the comparable periods in 2021, and consisted of research and development expenses and general and administrative expenses.
- Research and development expenses for the three and twelve months ended December 31, 2022 were $27.2 million and $98.4 million, respectively, as compared to $23.6 million and $93.1 million for the comparable periods in 2021. Research and development expenses increased by $5.3 million in 2022 primarily due to increases in direct costs of $9.3 million related to pheEDIT and $3.9 million related to juMPStart, as we incurred costs to initiate sites and recruit patients. Additionally, there was a $5.8 million increase in direct research expenses related to our other development-stage programs, primarily due to higher spending on HMI-104. Partially offsetting these increases was a $15.6 million decrease in employee-related costs as a result of transferring employees to OXB Solutions in order to leverage the Company's in-house manufacturing capabilities while establishing a 20% ownership stake and preferred customer status in the new business.
- General and administrative expenses for the three and twelve months ended December 31, 2022 were $8.1 million and $38.1 million, respectively, as compared to $10.8 million and $36.8 million for the comparable periods in 2021. General and administrative expenses increased in 2022 due primarily to professional fees associated with the establishment of OXB Solutions.
- As of December 31, 2022, Homology had approximately $175.0 million in cash, cash equivalents and short-term investments. Based on current projections, Homology expects current cash resources to fund operations into the fourth quarter of 2024.

Upcoming Events

- Project Alive Hunter Syndrome Community Conference: March 11 at 2:30 p.m. ET
- 2023 ACMG Annual Clinical Genetics Meeting: March 14 - 18

About Homology Medicines, Inc.
Homology Medicines, Inc. is a clinical-stage genetic medicines company dedicated to transforming the lives of patients suffering from rare diseases by addressing the underlying cause of the disease. The Company's clinical programs include HMI-103, a gene editing candidate for phenylketonuria (PKU); HMI-203, an investigational gene therapy for Hunter syndrome; and HMI-102, an investigational gene therapy for adults with PKU. Additional programs focus on paroxysmal nocturnal hemoglobinuria (PNH), metachromatic leukodystrophy (MLD) and other diseases. Homology's proprietary platform is designed to utilize its family of 15 human hematopoietic stem cell-derived adeno-associated virus (AAVHSCs) vectors to precisely and efficiently deliver genetic medicines *in vivo* through a nuclease-free gene editing modality, gene therapy, or GTx-mAb, which is designed to produce

antibodies throughout the body. Homology established an AAV manufacturing and innovation business in partnership with Oxford Biomedica, which was based on Homology's internal process development and manufacturing platform. Homology has a management team with a successful track record of discovering, developing and commercializing therapeutics with a focus on rare diseases. Homology believes its initial clinical data and compelling preclinical data, scientific and product development expertise and broad intellectual property position the Company as a leader in genetic medicines. For more information, visit www.homologymedicines.com.

Forward-Looking Statements

This press release contains forward-looking statements. We intend such forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. All statements contained in this press release that do not relate to matters of historical fact should be considered forward-looking statements, including without limitation statements regarding: our plans to engage in future collaborations and strategic partnerships; our expectations surrounding the potential, safety, efficacy, and regulatory and clinical progress of our product candidates; the potential of our gene therapy and gene editing platforms, including our GTx-mAb platform; our plans and timing for the release of additional preclinical and clinical data; our plans to progress our pipeline of genetic medicine candidates and the anticipated timing for these milestones; our position as a leader in the development of genetic medicines; the sufficiency of our cash and cash equivalents to fund our operations; and our participation in upcoming presentations and conferences. The words "believe," "may," "will," "estimate," "potential," "continue," "anticipate," "intend," "expect," "could," "would," "project," "plan," "target," and similar expressions are intended to identify forward-looking statements, though not all forward-looking statements use these words or expressions. These statements are neither promises nor guarantees, but involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements, including, but not limited to, the following: we have and expect to continue to incur significant losses; our need for additional funding, which may not be available; failure to identify additional product candidates and develop or commercialize marketable products; the early stage of our development efforts; potential unforeseen events during clinical trials could cause delays or other adverse consequences; risks relating to the regulatory approval process; interim, topline and preliminary data may change as more patient data become available, and are subject to audit and verification procedures that could result in material changes in the final data; our product candidates may cause serious adverse side effects; inability to maintain our collaborations, or the failure of these collaborations; our reliance on third parties, including for the manufacture of materials for our research programs, preclinical and clinical studies; failure to obtain U.S. or international marketing approval; ongoing regulatory obligations; effects of significant competition; unfavorable pricing regulations, third-party reimbursement practices or healthcare reform initiatives; product liability lawsuits; securities class action litigation; the impact of the COVID-19 pandemic and general economic conditions on our business and operations, including our preclinical studies and clinical trials; failure to attract, retain and motivate qualified personnel; the possibility of system failures or security breaches; risks relating to intellectual property; and significant costs incurred as a result of operating as a public company. These and other important factors discussed under the caption "Risk Factors" in our Annual Report on Form 10-K for the year ended December 31, 2022 and our other filings with the Securities and Exchange Commission could cause actual results to differ materially from those indicated by the forward-looking statements made in this press release. Any such forward-looking statements represent management's estimates as of the date of this press release. While we may elect to update such forward-looking statements at some point in the future, we disclaim any obligation to do so, even if subsequent events cause our views to change.

- Financial Tables Follow -

HOMOLOGY MEDICINES, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(in thousands)

	December 31,	
	2022	**2021**
Cash, cash equivalents and short-term investments	$ 175,026	$ 155,873
Assets held for sale	—	28,907
Equity method investment	25,814	—
Property and equipment, net	1,078	2,252
Right-of-use assets	20,563	15,607
Other assets	5,989	9,082
Total assets	$ 228,470	$ 211,721
Accounts payable, accrued expenses and other liabilities	$ 19,859	$ 13,772
Operating lease liabilities	1,561	246
Operating lease liabilities, net of current portion	27,916	23,688
Deferred revenue	1,156	4,364
Stockholders' equity	177,978	169,651
Total liabilities and stockholders' equity	$ 228,470	$ 211,721

HOMOLOGY MEDICINES, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS
(in thousands, except share and per share amounts)

	For the Three Months Ended December 31,		For the Year Ended December 31,	
	2022	**2021**	**2022**	**2021**
	(unaudited)			

Collaboration revenue	$ 802	$ 802	$ 3,208	$ 33,971
Operating expenses:				
Research and development	27,149	23,646	98,351	93,085
General and administrative	8,147	10,781	38,138	36,835
Total operating expenses	35,296	34,427	136,489	129,920
Loss from operations	(34,494)	(33,625)	(133,281)	(95,949)
Other income:				
Gain on sale of business	—	—	131,249	—
Interest income	1,455	42	3,230	185
Total other income	1,455	42	134,479	185
Income (loss) before income taxes	(33,039)	(33,583)	1,198	(95,764)
Benefit from (provision for) income taxes	101	—	(715)	—
Loss from equity method investment	(1,357)	—	(5,488)	—
Net loss	$ (34,295)	$ (33,583)	$ (5,005)	$ (95,764)
Net loss per share-basic and diluted	$ (0.60)	$ (0.59)	$ (0.09)	$ (1.73)
Weighted average common shares outstanding-basic and diluted	57,483,402	57,150,079	57,399,762	55,283,318

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Source: Homology Medicines, Inc.