Homology Medicines Reports Third Quarter 2022 Financial Results and Recent Highlights

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- On Track for Updates on pheEDIT and juMPStart Programs by Year-End -

- Strong Financial Position with Cash Runway into Fourth Quarter 2024 -

- Promoted Albert Seymour, Ph.D., to Chief Executive Officer Driving Clinical Trials and Genetic Medicines Platform Forward for Patients -

BEDFORD, Mass., Nov. 10, 2022 (GLOBE NEWSWIRE) -- Homology Medicines, Inc. (Nasdaq: FIXX), a genetic medicines company, announced today financial results for the third quarter ended September 30, 2022, and highlighted recent accomplishments.

"We remain on track to provide an update from our pheEDIT and juMPStart clinical trials, including site initiations and enrollment status, by the end of this year," said Albert Seymour, Ph.D., President and Chief Executive Officer of Homology Medicines. "With our recent pipeline prioritization, which included devoting resources to our ongoing clinical trials and select preclinical programs, Homology extended its cash runway into the fourth quarter of 2024 through key milestones across all programs. We continue to progress HMI-104, our lead GTx-mAb development candidate, through IND-enabling studies and believe that our optimized *in vivo* gene therapy development candidate for MLD, which has demonstrated the ability to cross the blood-brain-barrier following a single I.V. administration in preclinical studies, is differentiated from other approaches and makes it an attractive program for us to partner. Our work to bring first-in-class genetic medicine candidates forward for patients is ongoing, and we look forward to sharing our continued progress."

Third Quarter 2022 and Recent Highlights

- Announced a pipeline prioritization, which included shifting resources from the pheNIX gene therapy trial evaluating HMI-102 for phenylketonuria (PKU) and pausing enrollment to focus on generating data from the pheEDIT clinical trial that is evaluating *in vivo* gene editing candidate HMI-103 for PKU. The mechanism of action of this gene editing candidate has the potential to treat adults and ultimately pediatric patients.
- Continued to advance the juMPStart clinical trial evaluating *in vivo* gene therapy candidate HMI-203 for MPS II, or Hunter syndrome. Homology remains on track to provide an update on enrollment and site status for this trial and the pheEDIT trial by the end of this year.
- Continued to advance IND-enabling studies of HMI-104, a C5 antibody development candidate for paroxysmal nocturnal hemoglobinuria (PNH), which utilizes the Company's GTx-mAb platform that has the potential to address larger market indications.
- Announced intention to partner the optimized *in vivo* gene therapy candidate HMI-204 for metachromatic leukodystrophy (MLD), which demonstrated the ability to cross the blood-brain-barrier as well as reach the central nervous system and key peripheral organs involved in MLD following a single I.V. administration.
- Presented design of the pheEDIT gene editing trial for HMI-103 and supporting preclinical data, as well as data that demonstrated the use of next-generation sequencing methods that can lead to improved AAVHSC vector design, which were featured at the American Society of Human Genetics Meeting.
- Promoted Albert Seymour, Ph.D., to Chief Executive Officer of Homology and to the Board of Directors, succeeding Arthur Tzianabos, Ph.D., who served as CEO and Board member since 2016 and who was appointed Chair of the Board in conjunction with this transition.
- Announced peer-reviewed publication in *Scientific Reports*, a *Nature* journal, on the use of long-read sequencing methods that could be applied to further characterize Homology's AAVHSCs and optimize vector design.
- Spoke alongside industry leaders on panels focused on innovations in gene editing and AAV manufacturing during the Cell & Gene Meeting on the Mesa.

Third Quarter 2022 Financial Results

- As of September 30, 2022, Homology had approximately $201.1 million in cash, cash equivalents and short-term investments. Based on current projections, Homology expects cash resources to fund operations into the fourth quarter of 2024.
- Net loss for the quarter ended September 30, 2022 was $(33.7) million or $(0.59) per share, compared to a net loss of $(30.6) million or $(0.54) per share for the quarter ended September 30, 2021.
- Collaboration revenues for the quarter ended September 30, 2022 were $0.8 million, compared to $1.7 million for the quarter ended September 30, 2021. Collaboration revenue in each period included deferred revenue recognized under Homology's stock purchase agreement with Pfizer. Collaboration revenues for the third quarter 2021 also included the recognition of deferred revenue and reimbursement of R&D expenses under the Company's former collaboration with Novartis.
- Total operating expenses for the quarter ended September 30, 2022 were $33.7 million, compared to $32.3 million for the quarter ended September 30, 2021, and consisted of research and development expenses and general and administrative expenses.
- Research and development expenses for the quarter ended September 30, 2022 were $25.9 million, compared to $24.0 million for the quarter ended September 30, 2021. Research and development expenses increased primarily due to increased development costs for our ongoing HMI-103 and HMI-203 clinical-stage programs and earlier-stage programs. Partially offsetting these increases were lower employee-related costs as a result of transferring employees to Oxford Biomedica Solutions, the AAV Innovation and Manufacturing Business that Homology established with Oxford Biomedica in the first quarter of 2022 in order to leverage its in-house manufacturing capabilities while establishing a 20% stake and preferred customer status in the new business.
- General and administrative expenses for the quarter ended September 30, 2022 were $7.8 million, compared to $8.4 million for the quarter ended September 30, 2021. General and administrative expenses decreased primarily as a result of finance, human resources, IT and legal services provided by the Company to OXB Solutions under the transitional services agreement that provided for these services to be reimbursed, as well as decreased depreciation expense as the Company's leasehold improvements were transferred to Oxford Biomedica Solutions. Partially offsetting these decreases was an increase in audit and legal fees.

Upcoming Events

- Stifel 2022 Healthcare Conference: November 15, 2022 at 4:10 p.m. ET – Fireside Chat

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-102, an investigational gene therapy for adults with phenylketonuria (PKU); HMI-103, a gene editing candidate for PKU; and HMI-203, an investigational gene therapy for Hunter syndrome. Additional programs focus on metachromatic leukodystrophy (MLD), paroxysmal nocturnal hemoglobinuria (PNH) 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 gene therapy or nuclease-free gene editing modality, as well as to deliver one-time gene therapy to produce antibodies throughout the body through the GTx-mAb 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 within the meaning of the Private Securities Litigation Reform Act of 1995. 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 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 position as a leader in the development of genetic medicines; the sufficiency of our cash and cash equivalents to fund our operations; our plans to engage in future

collaborations and strategic partnerships; 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: the impact of the COVID-19 pandemic on our business and operations, including our preclinical studies and clinical trials, and on general economic conditions; 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; 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 Quarterly Report on Form 10-Q for the quarter ended September 30, 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)
(Unaudited)

	As of	
	September 30, 2022	**December 31, 2021**
Cash, cash equivalents and short-term investments	$ 201,074	$ 155,873
Assets held for sale	—	28,907
Equity method investment	27,132	—
Property and equipment, net	1,415	2,252
Right-of-use assets	20,900	15,607
Other assets	12,130	9,082
Total assets	$ 262,651	$ 211,721
Accounts payable, accrued expenses and other liabilities	$ 21,805	$ 13,772
Operating lease liabilities	29,827	23,934
Deferred revenue	1,958	4,364
Stockholders' equity	209,061	169,651
Total liabilities and stockholders' equity	$ 262,651	$ 211,721

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

	Three months ended September 30,		Nine months ended September 30,	
	2022	**2021**	**2022**	**2021**
Collaboration revenue	$ 802	$ 1,677	$ 2,406	$ 33,169
Operating expenses:				
Research and development	25,854	23,987	71,202	69,439
General and administrative	7,810	8,351	29,991	26,054
Total operating expenses	33,664	32,338	101,193	95,493
Loss from operations	(32,862)	(30,661)	(98,787)	(62,324)
Other income:				
Gain on sale of business	-	—	131,249	—
Interest income	1,269	53	1,775	143
Total other income	1,269	53	133,024	143
Income (loss) before income taxes	(31,593)	(30,608)	34,237	(62,181)
Benefit from (provision for) income taxes	46	—	(816)	—
Loss from equity method investment	(2,179)	—	(4,131)	—
Net income (loss)	$ (33,726)	$ (30,608)	$ 29,290	$ (62,181)
Net income (loss) per share-basic	$ (0.59)	$ (0.54)	$ 0.51	$ (1.14)

Net income (loss) per share-diluted	$ (0.59)	$ (0.54)	$ 0.51	$ (1.14)
Weighted-average common shares outstanding-basic	57,447,192	57,106,639	57,372,399	54,704,410
Weighted-average common shares outstanding-diluted	57,447,192	57,106,639	57,901,298	54,704,410

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