Homology Medicines Announces $60 Million Equity Investment from Pfizer Inc.

BEDFORD, Mass., November 9, 2020 – Homology Medicines, Inc. (Nasdaq: FIXX), a genetic medicines company, announced today that Pfizer Inc. (NYSE: PFE) has agreed to make a $60 million equity investment in Homology. Pfizer has agreed to purchase 5,000,000 of Homology's common stock at a price of $12.00 per share, as part of the Pfizer Breakthrough Growth Initiative, which was announced earlier this year. The purchase by Pfizer is expected to close on or about November 10, 2020.

"With the positive clinical data from the dose-escalation phase of our pheNIX Phase 1/2 trial for adults with phenylketonuria (PKU) and our plans to move to the expansion phase of the trial, both of which we announced last week, we believe Pfizer's investment in Homology is a testament to their enthusiasm for our PKU gene therapy and gene editing programs to treat people living with this disease," stated Arthur Tzianabos, Ph.D., President and Chief Executive Officer of Homology Medicines.

"Pfizer Rare Disease has a 30-year heritage of developing treatment options for patients with some of the greatest unmet needs, including in the area of rare metabolic disorders," said Seng Cheng, Ph.D., Senior Vice President and Chief Scientific Officer of Pfizer's Rare Disease Research Unit. "Our investment in Homology represents another example of our commitment to collaborate with the biotechnology community. We believe gene therapy could help provide a potentially transformational therapeutic option for patients living with PKU and is a good strategic fit with our rAAV-associated gene therapy portfolio."

In connection with the investment, Pfizer's Dr. Cheng will join Homology's Scientific Advisory Board to participate in matters related to the development of the Company's PKU product candidates: HMI-102 gene therapy candidate for adults with PKU and HMI-103 gene editing candidate for pediatric patients with PKU. Additionally, the Company has granted Pfizer a right of first refusal on future transactions involving these programs.

Homology intends to use the net proceeds of the offering to help fund its ongoing and planned PKU clinical trials, as well as the Company's central nervous system (CNS) programs. Based on current projections, together with the anticipated proceeds of $60 million from the Pfizer equity investment, Homology expects cash resources to fund operations into the third quarter of 2022.

About HMI-102 Gene Therapy and HMI-103 Gene Editing Product Candidates

HMI-102 is an investigational gene therapy in clinical development for the treatment of phenylketonuria (PKU) in adults. HMI-102 is designed to encode the *PAH* gene, which is mutated in people with PKU, delivered via the liver-tropic AAVHSC15 vector. Homology has received Fast Track Designation and orphan drug designation for HMI-102 from the U.S. Food and Drug Administration (FDA), and orphan drug designation from the European Medicines Agency (EMA). HMI-103 is an investigational, nuclease-free gene editing product candidate in IND-enabling studies for pediatric patients with PKU, whose livers are rapidly dividing. By delivering a functional copy of the *PAH* gene to replace the mutated copy in the genome, gene editing is the best approach for a potential one-time treatment for this population.

About Phenylketonuria (PKU)

PKU is a rare inborn error of metabolism caused by a mutation in the *PAH* gene. PKU results in a loss of function of the enzyme phenylalanine hydroxylase, which is responsible for the metabolism of phenylalanine (Phe), an amino acid obtained exclusively from the diet. If left untreated, toxic levels of Phe can accumulate in the blood and result in progressive and severe neurological impairment. Currently, there are no treatment options for PKU that target the underlying genetic cause of the disease. According to the National PKU Alliance, PKU affects nearly 16,500 people in the U.S. with approximately 350 newborns diagnosed each year. The worldwide prevalence of PKU is estimated to be 50,000 people.

About Homology Medicines, Inc.

Homology Medicines, Inc. is a genetic medicines company dedicated to transforming the lives of patients suffering from rare genetic diseases with significant unmet medical needs by curing the underlying cause of the disease. Homology's proprietary platform is designed to utilize its human hematopoietic stem cell-derived adeno-associated virus vectors (AAVHSCs) to precisely and efficiently deliver genetic medicines *in vivo* either through a gene therapy or nuclease-free gene editing modality across a broad range of genetic disorders. Homology has a management team with a successful track record of discovering, developing and commercializing therapeutics with a particular focus on rare diseases, and intellectual property covering its suite of 15 AAVHSCs. Homology believes that its compelling preclinical data, scientific expertise, product development strategy, manufacturing capabilities and intellectual property position it as a leader in the development of genetic medicines. For more information, please 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. 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 expectation regarding the timing for the closing of the Pfizer investment; our planned use of proceeds of the Pfizer investment; the sufficiency of our cash resources, together with the anticipated proceeds of $60 million from the Pfizer equity investment, to fund our operations; our expectations surrounding the potential, safety, efficacy, and regulatory and clinical progress of our product candidates; plans and timing surrounding the Phase 1/2 pheNIX trial, including the expansion phase and the

potential for conversion to a registrational trial; and our position as a leader in the development of genetic medicines. 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 capabilities of our manufacturing facility; 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; 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; failure to attract, retain and motivate qualified personnel; the possibility of system failures or security breaches; risks relating to intellectual property and significant costs 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 quarterly period ended June 30, 2020 and our other filings with the SEC 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.

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