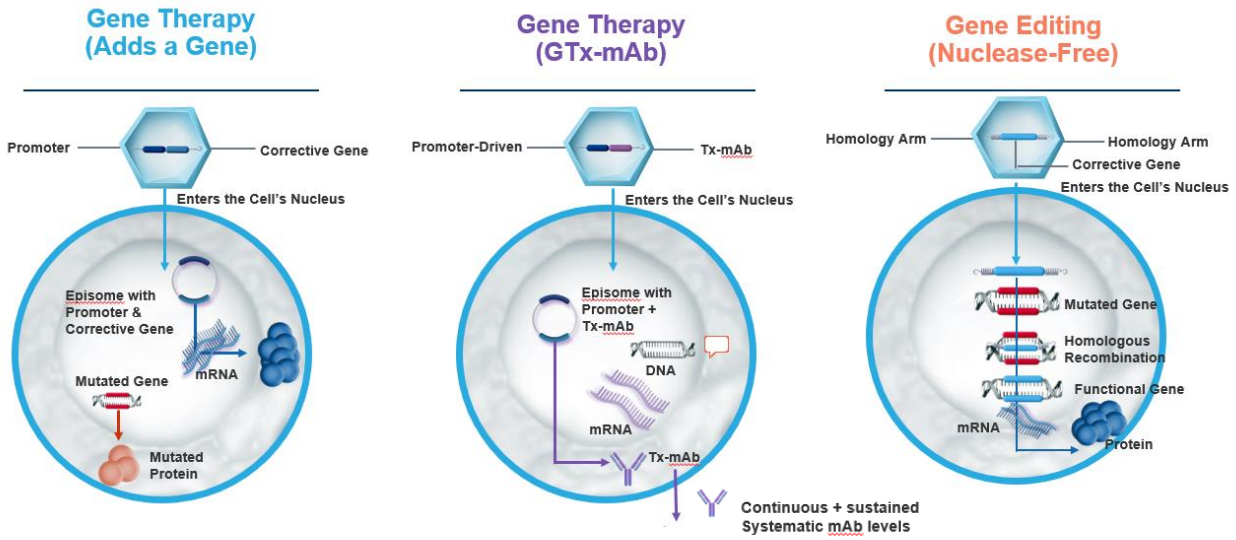
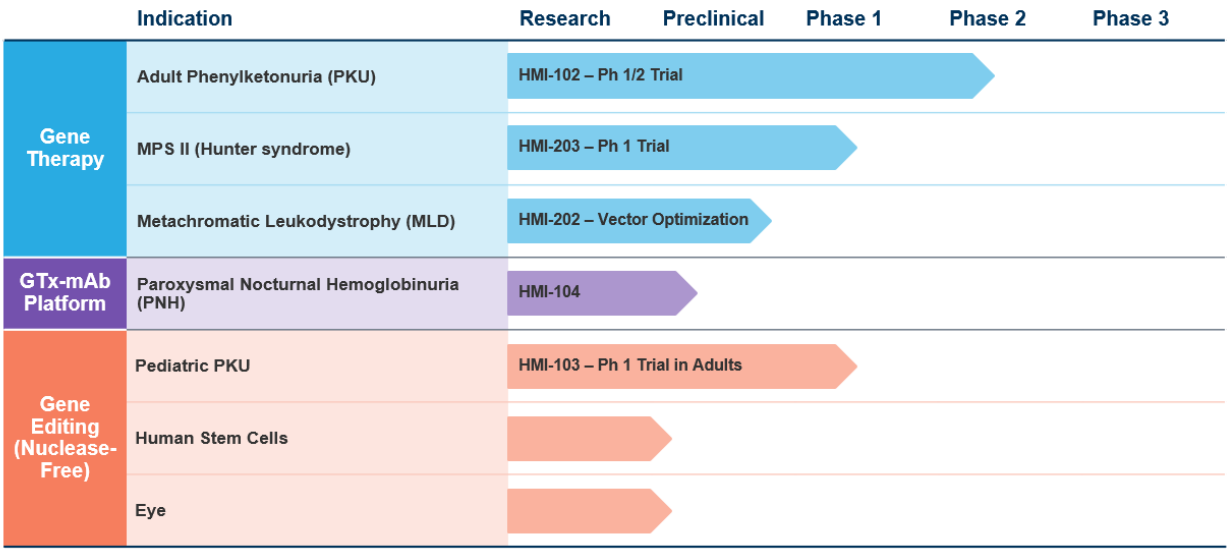
HOMOLOGY
Medicines, Inc.

Homology Medicines, Inc. (Nasdaq: FIXX) is a clinical-stage genetic medicines company translating its broad *in vivo* platform into potential one-time treatments, or cures, for the rare disease community.

Homology's technology is based on its proprietary family of 15 adeno-associated virus vectors derived from human stem cells (AAVHSCs). AAVHSCs have the potential to deliver one-time genetic medicines *in vivo* through a **gene therapy** or nuclease-free **gene editing** approach, as well as deliver gene therapy to produce antibodies throughout the body through the **GTx-mAb** platform.



Homology is developing one-time treatments for rare diseases, including PKU, an inherited metabolic disorder, MPS II, a lysosomal storage disorder, and multiple discovery-stage programs such as PNH, a rare blood disorder.



1 Patriots Park, Bedford, MA 01730 | 781-301-7277 | www.homologymedicines.com

Homology's Clinical Programs for 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. PKU affects* nearly 16,500 people in the U.S. with approximately 350 newborns diagnosed each year.

**National PKU Alliance*

pheEDIT

HMI-103 is designed as a one-time nuclease-free gene editing candidate that is being evaluated in the pheEDIT clinical trial, a Phase 1 dose-escalation study for adults with PKU.

Over time, Homology plans to evaluate HMI-103 in the pediatric PKU population. Homology plans to provide an update on this trial by year-end.

HMI-103 was granted Fast Track designation by the U.S. Food and Drug Administration.

pheNIX
CLINICAL STUDY FOR PATIENTS WITH PKU

HMI-102 is designed as a one-time gene therapy candidate that is being evaluated in the pheNIX clinical trial, a Phase 1/2 study for adults with PKU.

HMI-102 was granted Orphan Drug Designation and Fast Track Designation by the U.S. FDA and Orphan Drug Designation by the EMA.

Homology's Clinical Program for MPS II

MPS II is a rare, X-linked lysosomal storage disorder caused by mutations in the *IDS* gene, which is responsible for producing the I2S enzyme that breaks down glycosaminoglycans (GAGs). Severe MPS II results in toxic lysosomal accumulation of GAGs that causes progressive debilitation and decline in intellectual function. MPS II occurs in approximately 1 in 100,000 to 1 in 170,000 males.

juMPStart

HMI-203 is designed as a one-time gene therapy that is being evaluated in the juMPStart clinical trial, a Phase 1 dose-escalation trial for adults with MPS II. Homology plans to provide an update on this trial by year-end.

HMI-203 was granted Orphan Drug Designation by the U.S. FDA.

New Manufacturing and Innovation Business, Oxford Biomedica Solutions

Homology formed a new Manufacturing and Innovation Business with UK-based, global viral vector manufacturer Oxford Biomedica, which incorporates Homology's leading internal technical and manufacturing operations. As a 20% owner of Oxford Biomedica Solutions, Homology has access to the AAV 'plug and play' process and platform that met CMC requirements for its three INDs. As a preferred customer, Homology is continuing to work with AAVHSC experts within the state-of-the-art manufacturing facility built by Homology.

Homology's Senior Leadership Team

Homology has a team of experienced and dedicated leaders in rare diseases and genetic medicines with a proven track record of developing and launching rare disease drugs.