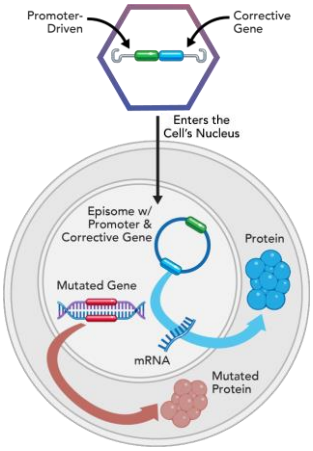
HOMOLOGY
Medicines, Inc.

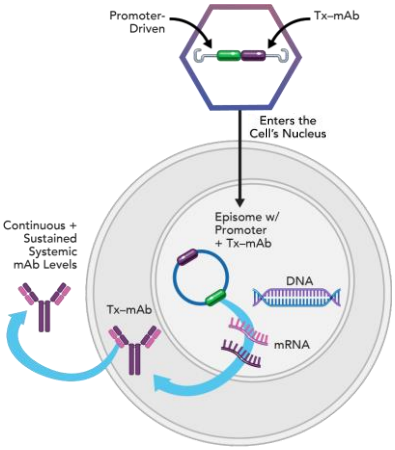
Homology Medicines, Inc. (Nasdaq: FIXX) is a clinical-stage genetic medicines company translating its broad *in vivo* platform into potential one-time treatments, or cures, for the rare disease community.

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.

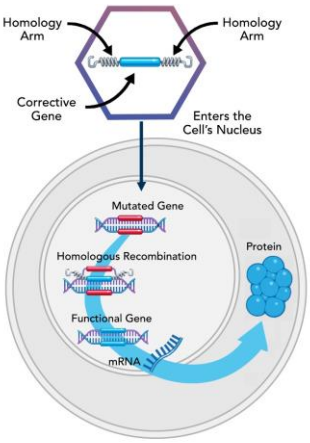
Gene Therapy
(Adds a Gene)



Gene Therapy
(GTx-mAb)



Gene Editing
(Replaces a Gene without Cutting DNA)



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.

	Indication	Research	Preclinical	Phase 1/2	Phase 3
Gene Therapy	Adult Phenylketonuria (PKU)	HMI-102 – Initial Ph 2 Data Expected Mid-2022			
	MPS II (Hunter syndrome)	HMI-203 – Ph 1/2 Trial Initiation Expected 2021			
	Metachromatic Leukodystrophy (MLD)	HMI-202 – Vector Optimization			
GTx-mAb Platform	Paroxysmal Nocturnal Hemoglobinuria (PNH)	HMI-104			
Gene Editing (Nuclease-Free)	Pediatric PKU	HMI-103 – Ph 1 Trial			
	Human Stem Cells				
	Eye				

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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) from protein. 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*

pheNIX
CLINICAL STUDY FOR PATIENTS WITH PKU

pheNIX:

HMI-102 is a potential one-time gene therapy that is being evaluated in the **Phase 2 dose expansion**, concurrently controlled portion of the pheNIX clinical trial for adults with PKU. Sites are enrolling patients in pheNIX and initial data are expected from the dose expansion phase by mid-2022. Learn more at **phenixpku.com**.

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

pheEDIT:

pheEDIT is a Phase 1 dose-escalation clinical trial evaluating nuclease-free gene editing candidate HMI-103 for adults with PKU. Over time, Homology plans to evaluate HMI-103 in the pediatric PKU population.

Homology's Manufacturing Expertise

Homology has a commercial GMP manufacturing platform and process, including a 25,000-sq-ft facility in Bedford, Mass., with capacity to supply its gene therapy and gene editing programs. Homology was one of the first companies to scale to a 2,000L bioreactor in an HEK293 suspension system and has 3,500L active capacity. Homology leverages a "plug and play" model to allow for efficient changes to capsids or cDNA and scaling to the clinic and potentially beyond.

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/or launching 11 rare disease drugs. Learn more at **homologymedicines.com/about.**

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