BioMarin and hemophilia timeline

KEY: HEMOPHILIA · PIONEERS · GENETICS · BIOMARIN

1800s

Hemophilia—The royal disease[1]
1800s

Mendel's theory of inheritance[2]
1865

DNA isolated[3]
1869

1940s-1950s

Whole-blood transfusion in hospital[4]
1940s

DNA double helix described[3,5,6]
1953

1970s

At-home replacement therapy available, with plasma-derived factor concentrates[7]
1970s

Dr. Barrie Carter begins work on AAV-mediated gene transfer biotechnology at the National Institutes of Health
1970

Dr. Gordon Vehar begins work on factor VIII
1976

1980s

Genes for factor VIII and factor IX are cloned[8]
1980s

The Centers for Disease Control (CDC) reports first AIDS case in hemophilia[9]
1982

Dr. Barrie Carter publishes a paper describing the use of AAV as a vector[10]
1984

Dr. Gordon Vehar publishes a paper reporting successful factor VIII cloning[11]
1984

Dr. Wing Yen Wong begins hematology fellowship
1987

1990s

First gene therapy trial in humans[12]
1990

Recombinant clotting factors approved
FVIII 1992[1]
FIX 1997[1]
FVIIa 1999[13]

BIOMARIN

BioMarin incorporates[15]
1997

Lessons learned regarding risks related to potential for severe immune response in early gene therapy trial with non-AAV vector[16]
1999

2000s

Prophylaxis becomes standard of care[17]
2000s

Human Genome Project completed[18]
2003

First gene therapy trial in hemophilia B using AAV vector technology[19]
2005

BioMarin therapies for rare diseases approved
Mucopolysaccharidosis I
2003[20]
Mucopolysaccharidosis VI
2005[21]
Phenylketonuria
2007[22]

Dr. Gordon Vehar joins BioMarin
2008

2010s

Dr. Barrie Carter joins BioMarin
2011

Extended half-life factors approved[23]
2014

BioMarin therapies for rare diseases approved
Mucopolysaccharidosis IVA
2014[24]
Neuronal ceroid lipofuscinosis type 2
2017[25]

Investigational gene therapy by BioMarin: Hemophilia A trial to research safety and efficacy starts (BMN 270-201)[26]
2015

Dr. Wing Yen Wong joins BioMarin
2016

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BIOMARIN hemophilia