

# BioMarin Haemophilia Timeline

KEY: ● HAEMOPHILIA ● PIONEERS ● GENETICS ● BIOMARIN

1800s

**Haemophilia—  
The royal disease**  
1800s

**Mendel's theory of  
inheritance**  
1865

**DNA isolated**  
1869

1940s-1950s

**Whole-blood transfusion  
in hospital**  
1940s

**DNA double helix described**  
1953

1970s

**At-home replacement  
therapy available, with  
plasma-derived factor  
concentrates**  
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**  
1980s

**The Centers for Disease  
Control (CDC) reports first  
AIDS case in haemophilia**  
1982

**Dr Barrie Carter publishes  
a paper describing the use  
of AAV as a vector**  
1984

**Dr Gordon Vehar publishes  
a paper reporting  
successful factor VIII  
cloning**  
1984

**Dr Wing Yen Wong begins  
haematology fellowship**  
1987

1990s

**First gene therapy trial  
in humans**  
1990

**Recombinant clotting  
factors approved**  
FVIII 1992  
FIX 1997  
FVIIa 1999

**BIOMARIN**

**BioMarin incorporates**  
1997

**Lessons learned regarding  
risks related to potential  
response in early gene  
therapy trial with non-AAV  
vector**  
1999

2000s

**Prophylaxis is  
recommended as  
standard of care**  
2000s

**Human Genome Project  
completed**  
2003

**First gene therapy trial  
in haemophilia B using AAV  
vector technology**  
2005

**BioMarin therapies for  
rare diseases approved**  
Mucopolysaccharidosis I  
2003

Mucopolysaccharidosis VI  
2005

Phenylketonuria  
2007

Lambert-Eaton myasthenic  
syndrome  
2009

**Dr Gordon Vehar joins  
BioMarin**  
2008

2010s

**Dr Barrie Carter joins  
BioMarin**  
2011

**Extended half-life factors  
approved**  
2014

**BioMarin therapies for rare  
diseases approved**

Mucopolysaccharidosis IVA  
2014

Neuronal ceroid lipofuscinosis  
type 2  
2017

**Investigational gene  
therapy by BioMarin:  
Haemophilia A trial to  
research safety and  
efficacy starts  
(BMN 270-201)**  
2015

**Dr Wing Yen Wong joins  
BioMarin**  
2016

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