BIOMARIN MARKETED AND PIPELINE PROGRAMS

		PRECLINICAL TESTING	PHASE 1	PHASE 2	PHASE 3	BLA/NDA/MAA FILED	COMMERCIAL
MARKETED PROGRAMS*	Laronidase for MPS I**						
	Galsulfase for MPS VI			•			
	Sapropterin Dihydrochloride for PKU			•			
	Elosulfase Alfa for Morquio A Syndrome (MPS IVA)		•				
	Cerliponase Alfa for CLN2, a form of Batten Disease		•				
	Pegvaliase for PKU		0	•			
R&D PROGRAMS	Valoctocogene Roxaparvovec AAV-Gene Therapy for (BMN 270) Hemophilia A					•	
	Vosoritide (BMN 111) Analog of CNP for Achondroplasia				•	•	
	BMN 307 AAV-Gene Therapy for PKU			0 0 0 0 0 0			6 0 0 0 0 0 0 0 0 0 0 0 0 0 0 0 0 0 0 0
	BMN 255 Small molecule for a subset of chronic renal disease			0 0 0 0 0 0	•		0 0 0 0 0 0 0
	BMN 331 AAV-Gene Therapy for HAE		0 0 0 0 0 0	0 0 0 0 0 0	•		0 0 0 0 0
	BMN 351 Oligonucleotide for DMD		0 0 0 0				0 0 0 0 0 0
	DiNA-001 AAV-Gene Therapy for HCM***		0 0 0 0 0 0	0 0 0 0 0 0 0 0 0 0 0 0 0 0 0 0 0 0 0 0			0 0 0 0 0 0

^{*}Not all products in this category are approved for commercial use in all countries.

^{***}In collaboration with DiNAQOR



^{**}Laronidase was developed by BioMarin and licensed to Genzyme Europe B.V.