



# Development Pipeline

As of March 23<sup>rd</sup>, 2021

Code Generic name	Indication	Status	Mechanism	Remarks
JR-141 Pabinafusp Alfa	LSD   MPS type II (Hunter)	Japan: Approved; Brazil: Filed; Global: Phase III	BBB-penetrating iduronate-2 -sulfatase (rDNA origin)	<ul style="list-style-type: none"> <li>• ERT</li> <li>• J-Brain Cargo®</li> </ul>
JR-171	LSD   MPS type I (Hurler etc.)	Global: Phase I/II	BBB-penetrating α-L-iduronidase (rDNA origin)	<ul style="list-style-type: none"> <li>• ERT</li> <li>• J-Brain Cargo®</li> <li>• J-MIG System®</li> </ul>
JR-162	LSD   Pompe disease	Preclinical	J-Brain Cargo® -applied acid α-glucosidase (rDNA origin)	<ul style="list-style-type: none"> <li>• ERT</li> <li>• J-Brain Cargo®</li> </ul>
JR-441	LSD   MPS type III A (Sanfilippo A)	Preclinical	BBB-penetrating heparan N-sulfatase (rDNA origin)	<ul style="list-style-type: none"> <li>• ERT</li> <li>• J-Brain Cargo®</li> </ul>
JR-443	LSD   MPS type VII (Sly)	Preclinical	BBB-penetrating β-glucuronidase (rDNA origin)	<ul style="list-style-type: none"> <li>• ERT</li> <li>• J-Brain Cargo®</li> </ul>
JR-446	LSD   MPS type III B (Sanfilippo B)	Preclinical	BBB-penetrating α-N-acetylglucosaminidase (rDNA origin)	<ul style="list-style-type: none"> <li>• ERT</li> <li>• J-Brain Cargo®</li> </ul>
JR-401X	SHOX deficiency	Phase III	Human Growth hormone (rDNA origin)	<ul style="list-style-type: none"> <li>• Expanded Indication</li> </ul>
JR-142	Pediatric growth hormone deficiency	Phase II	Long-acting human growth hormone(rDNA origin)	<ul style="list-style-type: none"> <li>• J-MIG System®</li> </ul>
JR-041	Infertility	Phase I/II	Follicle stimulating hormone (rDNA origin)	<ul style="list-style-type: none"> <li>• Out-licensed to ASK Pharmaceutical Co., Ltd.</li> </ul>
JR-031HIE	Neonatal Hypoxic Ischemic Encephalopathy	Phase I/II	Human (Allogeneic) Bone Marrow Derived Mesenchymal Stem Cells	<ul style="list-style-type: none"> <li>• Expanded Indication</li> <li>• Regenerative medical product</li> </ul>
JTR-161 / JR-161	Acute cerebral infarction	Phase I/II	Dental pulp stem cells	<ul style="list-style-type: none"> <li>• Co-developed with Teijin Limited</li> <li>• Regenerative medical product</li> </ul>

BBB: Blood Brain Barrier | LSD: Lysosomal storage disorder | MPS: Mucopolysaccharidosis | ERT: Enzyme replacement therapy

**JCR do Brasil Farmacêuticos LTDA.**