

Krystal Biotech Pipeline

	Product	Protein	Indication	Discovery	Preclinical	Phase 1/2	Phase 3	Commercial
Dermatology	 Vyjuvek™ beremagene geperpavec-svdt 5x10 ⁹ PFU/mL single-use vial	Type VII collagen	Dystrophic Epidermolysis Bullosa	→				FDA Approved May 2023
	KB105 ^{†‡•‡}	Transglutaminase 1 (TGM1)	TGM1-deficient ARCI	→				
	KB104 [‡]	Serine Peptidase Inhibitor Kazal Type 5 (SPINK5)	Netherton Syndrome	→				
Respiratory	KB407 ^{†‡‡}	Cystic Fibrosis Transmembrane Conductance Regulator (CFTR)	Cystic Fibrosis	→				
	KB408	Alpha-1 antitrypsin (AATD)	Alpha-1 Antitrypsin Deficiency	→				
Ophthalmology	Ophthalmic B-VEC	Type VII collagen	Ocular Complications from Dystrophic Epidermolysis Bullosa	→				
Oncology	KB707 [•]	IL2 + IL12	Solid Tumors (Intratumoral Administration)	→				
	KB707	IL2 + IL12	Solid Tumors of the Lung (Inhaled Administration)	→				

All pipeline compounds except VYJUVEK™ are investigational.
All pipeline compounds are wholly owned.

†: FDA Orphan Drug Designation
‡: FDA Rare Pediatric Disease Designation
•: Fast-track Designation
‡: EMA Orphan Drug Designation

Rare disease

More prevalent conditions

Jeune Aesthetics Pipeline

	Product	Gene	Indication	Discovery	Preclinical	Phase 1	Phase 2	Phase 3	Commercial
Aesthetics	KB301	Type III collagen	TBD	→					
	KB302	Type I collagen	TBD	→					
	KB303	Elastin	TBD	→					
	KB304	Type III collagen & Elastin	TBD	→					
	KB305	Type IV collagen	TBD	→					

All pipeline compounds are investigational, being evaluated in clinical or pre-clinical studies.
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