

Russia List of Pediatric Rare Diseases and Orphan Drugs Covered by Special Fund				
Disease	Drug	Number of Children Per Year	Drug Cost Per Child Per Year (RUB)	Total Cost (RUB)
Short Bowel Syndrome	Takeda's Gattex (teduglutide)	91	18,900,000	1,700,000,000
	Novartis' Zykadia (ceritinib)	20	500,000	10,000,000
	Pfizer's Lorviqua (lorlatinib)	20	10,000,000	200,000,000
	EUSA's Qarziba (dinutuximab beta)	150	20,000,000	3,000,000,000
	Roche's Rozlytrek (entrectinib)	10	10,000,000	100,000,000
	Bristol-Myers Squibb's Sprycel (dasatinib)	5		
	Takeda's Inklusig (ponatinib)	5		
Malignant Neoplasms with NTRK Gene Mutation	Bayer's Vitrakvi (loratrektinib)	50	36,000,000	1,800,000,000
Brain Tumors	Novartis' Tafinlar (dabrafenib)	40	1,200,000	48,000,000
	Novartis' Mekinist (trametinib)	60	840,000	50,400,000
Catecholaminergic Polymorphic Ventricular Tachycardia	nadolol (no rug in Russia formulary)	32	8,000	256,000
Long QT Syndrome Jervel-Lange-Neilsen Syndrome	nadolol	100	8,000	800,000
	Actelion's Uptravi (selexipag)	10	2,400,000	24,000,000
	sildenafil	178	170,400	30,300,000
	Bayer's Ventavis (iloprost)	5	1,200,000	5,600,000
Congenital Hyperinsulinism	diazoxide	114		17,700,000
Hyper IgD Syndrome/Mevalonate Kinase Deficiency Syndrome (HIDS/MKD)	Novartis' Ilaris (canakinumab)	21	24,000,000	504,000,000
Cryopyrin-Associated Periodic Syndrome (CAPS)	Novartis' Ilaris (canakinumab)	48	12,000,000	576,000,000
	Roche's Actemra (tocilizumab)	10	2,000,000	20,000,000

	Roche's Actemra (tocilizumab)	20	2,000,000	40,000,000
	adalimumab	15	937,000	14,100,000
	etanercept	10	754,000	7,500,000
Familial Mediterranean Fever	Novartis' Ilaris (canakinumab)	120	12,000,000	1,400,000,000
	Roche's Actemra (tocilizumab)	15	2,000,000	30,000,000
	adalimumab	10	937,000	9,400,000
	etanercept	10	754,000	7,500,000
Pompe Disease	alglucosidase alfa	32	18,400,000	588,800,000
Hypophosphatasia (HPF)	asfotase alfa	15	38,700,000	579,800,000
Familial Hypercholesterolemia (Homozygous Form)	Amgen's Repatha (evolocumab) and lomitapide	20	24,500,000	490,000,000
Lysosomal Acid Lipase (LALL) Deficiency	Alexion's Kanuma (sebelipase alfa)	38	24,600,000	934,800,000
Mucopolysaccharidosis Type IV A	elosulfase alfa	39	38,600,000	1,500,000,000
Urea Cycle Disorder (UCM)	sodium glycerol butyrate and carboxylic acid	30	6,000,000	180,000,000
Spinal Muscular Atrophy (SMA)	Biogen's Spinraza (nusinersen) Roche's Evrysdi (risdiplam) Novartis' Zolgensma (onasemnogene abeparvovec)	890		23,000,000,000
Epilepsy and Epileptic Encephalopathy	tetracosactide hexaacetate	6,000	40,000	240,000,000
Tuberous Sclerosis	Novartis' Afinitor (everolimus)	425	4,000,000	1,700,000,000
Alpha Mannosidosis	velmanase alfa	16	25,000,000	400,000,000
Lipodystrophy	Amryt's Myalepta (metreleptin)	70	25,000,000	1,800,000,000
Duchenne Muscular Dystrophy (DMD)	PTC's Translarna (ataluren)	60	27,900,000	1,700,000,000
Neuronal Ceroid Lipofuscinosis Type 2	BioMarin's Brineura (cerliponase alfa)	30	56,000,000	1,700,000,000
Rickets	Kyowa Kirin's Crysvita (burosumab) and special phosphorous preparations	150	13,000,000	2,000,000,000
Violation of Beta-Oxidation of Fatty Acids (FAA)	triheptanoin	50	6,000,000	300,000,000
Phenylketonuria (PKU)	pegvaliase	150	2,800,000	420,000,000

Biotinidase Deficiency	biotin (high-dose not registered in Russia)	30	100,000	3,000,000
Bile Acid Synthesis Disorders	cholic acid	3	3,500,000	10,500,000
Leber Congenital Amaurosis	Novartis' Luxturna (voretigene neparvovec)	2	50,000,000	100,000,000