

List of the Compassionate Use programs of the Paul-Ehrlich-Institut

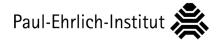
Last updated 21.02.2024

Medicinal Product: Lanadelumab

Active substance	Monoclonal antibody Lanadelumab
Pharmaceutical Form	Concentrate for solution for infusion
Application	Subcutaneous injection
Dosing	150 mg Lanadelumab, administered as a subcutaneous
	injection. 150 mg dose given either every 2 weeks or 4 weeks
Indication	Prevention of acute attacks of hereditary angioedema (HAE)
	in pediatric patients 2 to <12 years of age.
	To be eligible patients must have completed Study SHP643-
	301.
Date of Confirmed	29.01.2024
Notification	
Duration of	28.01.2025
Compassionate Use	
Programme	
Responsible Person	Responsible person:
(Company), Contact	Takeda Development Center Americas, Inc (TDC Americas)
Person	95 Hayden Avenue, Lexington, MA 02421, USA
	Contact person:
	Marcelo Freire
	Takeda Italia S.p.A
	Via Elio Vittorini 129
	00144 ROMA
	ITALIA
	Email: marcelo.freire@takeda.com
	Telephone: +41 79 758 4815

Medicinal Product: Elranatamab (PF-06863135)

Active substance	Elranatamab (PF-06863135) is a heterodimeric humanized
	full-length bispecific antibody against BCMA and CD3
Pharmaceutical Form	40 mg/mL solution for injection
Application	subcutaneous use
Dosing	Priming doses:
	12mg (cycle 1, day 1)
	32 mg (cycle 1, day 4)
	Full dose:
	76 mg/week (from cycle 1, day 8)



Indication	Monotherapy for the treatment of adult patients with relapsed and refractory multiple myeloma who have received at least three prior therapies, including an immunomodulatory agent, a proteasome inhibitor and an anti-CD38 monoclonal antibody, and have demonstrated disease progression on the last therapy.
Date of Confirmed	07.06.2023
Notification	
Duration of	06.06.2024
Compassionate Use	
Programme	
Responsible Person	Pfizer Inc.
(Company), Contact	235 East 42nd Street
Person	New York, NY 10017
	USA
	Local representative and contact details:
	Pfizer Pharma GmbH
	Dr. rer. medic. Claudia Herzberg
	Linkstraße 10
	10785 Berlin
	phone: +49 175 5706306
	e-mail: claudia.herzberg@pfizer.com

Medicinal Product: Talquetamab

Active substance	IgG-4 bispecific antibody targeting the CD3 receptor complex
	on T cells and GPRC5D on multiple myeloma cells
Pharmaceutical Form	Solution for injection
Application	subcutaneous administration
Dosing	Talquetamab will be administered using a body weight-based
	dosing-approach
	Step up-phase:
	day 1: 0,01 mg/kg
	day 3: 0,06 mg/kg*
	day 5: 0,4 mg/kg*
	day 7: 0,8 mg/ kg*
	Treatment-phase:
	0,8 mg/ kg* bi-weekly (q2w), minimum 12 days between
	doses
	* dose between 2-4 days (max. 7 days) after previous dose
	possible
Indication	As monotherapy for patients with relapsed and refractory
	multiple myeloma who have received at least three prior
	therapies, including a proteasome inhibitor, an
	immunomodulator and an anti-CD38 antibody
Date of Confirmed	23.03.2023



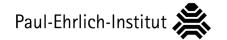
Notification	
Duration of	22.03.2024
Compassionate Use	
Programme	
Responsible Person	Janssen Pharmaceutica NV; Turnhoutseweg 30;
(Company), Contact	B-2340 Beerse,Belgium
Person	
	Contact in Germany:
	Janssen-Cilag GmbH,
	Medical Service Center
	E-Mail: jancil@its.jnj.com
	Tel.: +49 2137-955-955
	Fax.: +49 2137-955-443

Medicinal Product: rADAMTS13 (TAK-755)

Active substance	rADAMTS13 (TAK-755)
Pharmaceutical Form	Powder for solution for infusion
Application	intraveneous
Dosing	once weekly with 40 IU/kg [±4 IU/kg]
	prophylaxis dose of 40 IU/kg [±4 IU/kg] every 2 weeks*
	* Detailed information on the treatment regimen for special
	events is included in the program materials
Indication	Congenital thrombotic thrombocytopenic purpura
Date of Confirmed	21.02.2024
Notification	
Duration of	20.02.2025
Compassionate Use	
Programme	
Responsible Person	Takeda GmbH
(Company), Contact	Byk-Gulden-Straße 2
Person	78467 Konstanz
	Germany
	Contact:
	Stephan Regensburger
	Email: stephan.regensburger@takeda.com
	Tel.Nr. +4930206582238

Medicinal Product: Asunercept (APG101)

Active substance	Asunercept (APG101), a recombinant glycosylated fusion
	protein



Pharmaceutical Form	Concentrate for solution for infusion
Application	Intravenous infusion
Dosing	400 mg weekly
Indication	Newly diagnosed glioblastoma
Date of Confirmed	23.06.2023
Notification	
Duration of	22.06.2024
Compassionate Use	
Programme	
Responsible Person	Apogenix GmbH
(Company), Contact	Dr. Eike Buss
Person	Im Neuenheimer Feld 584
	D - 69120 Heidelberg
	Phone: +49 6221-586080

Medicinal Product: Rozanolixizumab

Active substance	Rozanolixizumab
Pharmaceutical Form	Solution for Injection
Application	Subcutaneous [SC]
	infusion
Dosing	Rozanolixizumab is provided as a 140 mg/mL solution for injection. Rozanolixizumab is administered as weekly doses for 6 weeks by SC infusion, using an appropriate infusion pump device set at a flow rate up to 20 mL/hour. The recommended total weekly dose of rozanolixizumab is dependent on the participants body weight.
	A treatment cycle consists of 1 dose per week for 6 weeks
	with subsequent cycles administered if and when the treating
	physician considers it appropriate for the participant.
Indication	Treatment of generalized myasthenia gravis (gMG) in
	symptomatic adult participants
Date of Confirmed	29.08.2023
Notification	
Duration of	28.08.2024
Compassionate Use	
Programme	
Responsible Person	UCB Pharma GmbH
(Company), Contact	Alfred-Nobel-Straße 10
Person	40789 Monheim
	Deutschland
	Contact Dr. Karl-Werner Leffers Email: karl-werner.leffers@ucb.com Telephone: +49-2173-48 1445



Medicinal Product: Efanesoctocog alfa

Active substance	Efanesoctocog alfa
Pharmaceutical Form	Powder and solvent for solution for injection
Application	Intravenous application
Dosing	The prophylactic treatment regimen is once weekly (every 7
	days) dosing with 50 IU/kg Efanesoctocog alfa.
Indication	Treatment and prophylaxis of bleeding in patients with
	haemophilia A (congenital factor VIII deficiency).
Date of Confirmed	26.01.2024
Notification	
Duration of	25.01.2025
Compassionate Use	
Programme	
Responsible Person	Swedish Orphan Biovitrum GmbH
(Company), Contact	Fraunhoferstrasse 9a
Person	82152 Martinsried
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	Stockholm
	Sweden)
	Contact:
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	Andrea.duemichen@sobi.com

Medicinal Product: Zolbetuximab

Active substance	Zolbetuximab
Pharmaceutical Form	Powder for concentrate for solution for infusion.
Application	Intravenous use
Dosing	In combination with fluoropyrimidine- and platinum-containing chemotherapy, 800 mg/m2 of Zolbetuximab on Cycle 1 Day 1 followed by subsequent doses of 600 mg/m2 every 3 weeks* * Detailed information on the treatment regimen is included in the program materials
Indication	First-line treatment of adult patients with locally advanced unresectable or metastatic HER2-negative gastric or gastroesophageal junction (GEJ) adenocarcinoma whose tumors are Claudin (CLDN)18.2 positive.
Date of Confirmed Notification	01.02.2024
Duration of Compassionate Use Programme	31.01.2025



Responsible Person	Astellas Pharma Europe B.V.
(Company), Contact	Sylviusweg 62
Person	2333 BE Leiden
	The Netherlands
	Contact:
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	+49(0)89 454401

Kontakt: ct@pei.de