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

Last updated 21.02.2024

## Medicinal Product: Lanadelumab

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| **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.<br>To be eligible patients must have completed Study SHP643-301. |
| **Date of Confirmed Notification** | 29.01.2024 |
| **Duration of Compassionate Use Programme** | 28.01.2025 |
| **Responsible Person (Company), Contact Person** | Responsible person:<br>Takeda Development Center Americas, Inc (TDC Americas)<br>95 Hayden Avenue, Lexington, MA 02421, USA<br><br>Contact person:<br>Marcelo Freire<br>Takeda Italia S.p.A<br>Via Elio Vittorini 129<br>00144 ROMA<br>ITALIA<br>Email: marcelo.freire@takeda.com<br>Telephone: +41 79 758 4815 |

## Medicinal Product: Elranatamab (PF-06863135)

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| **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:<br>12mg (cycle 1, day 1)<br>32 mg (cycle 1, day 4)<br><br>Full dose:<br>76 mg/week (from cycle 1, day 8) |

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| **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 Notification** | 07.06.2023 |
| **Duration of Compassionate Use Programme** | 06.06.2024 |
| **Responsible Person (Company), Contact Person** | Pfizer Inc.<br>235 East 42nd Street<br>New York, NY 10017<br>USA<br><br>**Local representative and contact details:**<br>Pfizer Pharma GmbH<br>Dr. rer. medic. Claudia Herzberg<br>Linkstraße 10<br>10785 Berlin<br>phone: +49 175 5706306<br>e-mail: claudia.herzberg@pfizer.com |

## Medicinal Product: Talquetamab

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| **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<br>**Step up-phase:**<br>day 1: 0,01 mg/kg<br>day 3: 0,06 mg/kg*<br>day 5: 0,4 mg/kg*<br>day 7: 0,8 mg/ kg*<br>**Treatment-phase:**<br>0,8 mg/ kg* bi-weekly (q2w), minimum 12 days between doses<br>* 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 |

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

## Medicinal Product: rADAMTS13 (TAK-755)

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

## Medicinal Product: Asunercept (APG101)

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

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

## Medicinal Product: Rozanolixizumab

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| **Active substance** | Rozanolixizumab |
| **Pharmaceutical Form** | Solution for Injection |
| **Application** | Subcutaneous [SC]<br>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.<br><br>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 Notification** | 29.08.2023 |
| **Duration of Compassionate Use Programme** | 28.08.2024 |
| **Responsible Person (Company), Contact Person** | UCB Pharma GmbH<br>Alfred-Nobel-Straße 10<br>40789 Monheim<br>Deutschland<br><br>Contact<br>Dr. Karl-Werner Leffers<br>Email: karl-werner.leffers@ucb.com<br>Telephone: +49-2173-48 1445 |

## Medicinal Product: Efanesoctocog alfa

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| **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 Notification** | 26.01.2024 |
| **Duration of Compassionate Use Programme** | 25.01.2025 |
| **Responsible Person (Company), Contact Person** | Swedish Orphan Biovitrum GmbH<br>Fraunhoferstrasse 9a<br>82152 Martinsried<br>(Representative of Swedish Orphan Biovitrum AB, SE-112 76 Stockholm<br>Sweden)<br><br>Contact:<br>Andrea Dümichen<br>Mobile +49 151 162 412 07<br>Andrea.duemichen@sobi.com |

## Medicinal Product: Zolbetuximab

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| **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*<br>* 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 |

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| **Responsible Person (Company), Contact Person** | Astellas Pharma Europe B.V.<br>Sylviusweg 62<br>2333 BE Leiden<br>The Netherlands<br><br>Contact:<br>Medizinischer Informations-Service<br>med.de@astellas.com<br>+49(0)89 454401 |

**Kontakt:** ct@pei.de