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

Last update 09.04.2024

## 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: 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** | 21.02.2024 |

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| **Notification** | |
| **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 |
| **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: Sotatercept

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| **Active substance** | Fusion protein sotatercept |
| **Pharmaceutical Form** | Powder and solvent for solution for injection. |
| **Application** | subcutan |

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| **Dosing** | Sotatercept is administered once every 3 weeks by subcutaneous injection according to patient weight.<br>*Recommended starting dose*<br>The starting dose of Sotatercept is 0.3 mg/kg.<br>*Recommended target dose*<br>The target dose of Sotatercept is 0.7 mg/kg administered every 3 weeks. |
| **Indication** | Sotatercept is indicated for the treatment of pulmonary arterial hypertension (PAH) in adult patients on standard of care with WHO Functional Class (FC) II to III. |
| **Date of Confirmed Notification** | 20.12.2023 |
| **Duration of Compassionate Use Programme** | 19.12.2024 |
| **Responsible Person (Company), Contact Person** | MSD Sharp & Dohme GmbH,<br>Levelingstr. 4a, 81673, München, Germany<br>Contact: Medizinisches Informationszentrum<br>Tel: 0800 673 673 673<br>infocenter@msd.de |

## 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 |
| **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