**Apéndice**

**Tabla I**

Información sobre medicamentos huérfanos incluidos en el análisis

|  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- |
|  |  | **Medicamentos huérfanos autorizados por la EMA, con designación vigente a fecha 31/12/18**  **(n = 100)** | **Medicamentos huérfanos autorizados por la EMA, con designación vigente y con CN en España**  **(n = 86)** | | | **Medicamentos huérfanos autorizados por la EMA, con designación vigente y sin CN en España**  **(n = 14)** |
|  |  |  | **Medicamentos huérfanos autorizados por la EMA, con designación vigente y con CN en España, y comercializados en España**  **(n = 54)** | | **Medicamentos huérfanos autorizados por la EMA, con designación vigente y con CN en España, y no comercializados en España (n = 32)** |  |
|  |  |  | **Financiados**  **(n = 51)** | **No financiados**  **(n = 3)** |  |  |
| 1 | Brentuximab vedotin | Adcetris | X |  |  |  |
| 2 | Riociguat | Adempas | X |  |  |  |
| 3 | Eftrenonacog alfa | Alprolix |  | X |  |  |
| 4 | Ofatumumab | Arzerra | X |  |  |  |
| 5 | Avelumab | Bavencio |  |  | X |  |
| 6 | Inotuzumab ozogamicin | Besponsa |  |  | X |  |
| 7 | Blinatumomab | Blincyto |  |  | X |  |
| 8 | Bosutinib (as monohydrate) | Bosulif | X |  |  |  |
| 9 | Cerliponase alfa | Brineura |  |  |  | X |
| 10 | Mannitol | Bronchitola | X |  |  |  |
| 11 | Carglumic acid | Carbaglu | X |  |  |  |
| 12 | Aztreonam lysine | Cayston | X |  |  |  |
| X3 | Histamine dihydrochloride | Ceplene |  |  | X |  |
| 14 | Eliglustat | Cerdelga | X |  |  |  |
| 15 | Chenodeoxycholic acid | Chenodeoxycholic acid sigma-tau |  |  |  | X |
| 16 | Human coagulation factor X | Coagadex |  |  |  | X |
| 17 | Cabozantinib | Cometriq |  |  | X |  |
| 18 | Isavuconazole | Cresemba | X |  |  |  |
| 19 | Mercaptamine hydrochloride | Cystadrops |  |  | X |  |
| 20 | Decitabine | Dacogen | X |  |  |  |
| 21 | Daratumumab | Darzalex | X |  |  |  |
| 22 | Defibrotide | Defitelio |  |  |  | X |
| 23 | Delamanid | Deltyba |  |  | X |  |
| 24 | Dinutuximab beta | Dinutuximab beta Apeiron |  |  |  | X |
| 25 | Pirfenidone | Esbriet | X |  |  |  |
| 26 | Panobinostat lactate anhydrous | Farydak |  |  | X |  |
| 27 | Icatibant | Firazyr | X |  |  |  |
| 28 | Amifampridine | Firdapse (previously Zenas) |  |  | X |  |
| 29 | Migalastat hydrochloride | Galafold |  |  | X |  |
| 30 | Obinutuzumab | Gazyvaro | X |  |  |  |
| 31 | Para-aminosalicylic acid | Granupas |  |  | X |  |
| 32 | Tasimelteon | Hetlioz |  |  |  | X |
| 33 | Ex vivo expanded autologous human corneal epithelial cells containing stem cells | Holoclar |  | X |  |  |
| 34 | Ponatinib | Iclusig | X |  |  |  |
| 35 | Albutrepenonacog alfa | Idelvion |  | X |  |  |
| 36 | Ibrutinib | Imbruvica | X |  |  |  |
| 37 | Pomalidomide | Imnovid | X |  |  |  |
| 38 | Rufinamide | Inovelon | X |  |  |  |
| 39 | Ivacaftor | Kalydeco | X |  |  |  |
| 40 | Sebelipase alfa | Kanuma | X |  |  |  |
| 41 | Ketoconazole | Ketoconazole HRA |  |  |  | X |
| 42 | Cholic acid | Kolbam |  |  |  | X |
| 43 | Sapropterin dihydrochloride | Kuvan | X |  |  |  |
| 44 | Carfilzomib | Kyprolis | X |  |  |  |
| 45 | Olaratumab | Lartruvo | X |  |  |  |
| 46 | Chlormethine | Ledaga |  |  | X |  |
| 47 | Lenvatinib mesylate | Lenvima | X |  |  |  |
| 48 | Lutetium (X77Lu) oxodotreotide | Lutathera |  |  | X |  |
| 49 | Olaparib | Lynparza | X |  |  |  |
| 50 | Mifamurtide | Mepact | X |  |  |  |
| 51 | Plerixafor | Mozobil | X |  |  |  |
| 52 | Parathyroid hormone | Natpar |  |  |  | X |
| 53 | Sorafenib | Nexavar | X |  |  |  |
| 54 | Concentrate of proteolytic enzymes enriched in bromelain | NexoBrid | X |  |  |  |
| 55 | Ixazomib citrate | Ninlaro |  |  | X |  |
| 56 | Romiplostim | Nplate | X |  |  |  |
| 57 | Obeticholic acid | Ocaliva |  |  | X |  |
| 58 | Nintedanib | Ofev | X |  |  |  |
| 59 | Irinotecan hydrochloride trihydrate | Onivyde |  |  | X |  |
| 60 | Macitentan | Opsumit | X |  |  |  |
| 61 | Cholic acid | Orphacol | X |  |  |  |
| 62 | Recombinant human nerve growth factor (rhNGF) | Oxervate |  |  | X |  |
| 63 | Caffeine citrate | Peyona | X |  |  |  |
| 64 | Hydrocortisone | Plenadren |  |  | X |  |
| 65 | Mercaptamine bitartrate | Procysbi |  |  |  | X |
| 66 | Glycerol phenylbutyrate | Ravicti |  |  | X |  |
| 67 | Idebenone | Raxone |  |  |  | X |
| 68 | Teduglutide | Revestive | X |  |  |  |
| 69 | Lenalidomide | Revlimid | X |  |  |  |
| 70 | Midostaurin | Rydapt |  |  | X |  |
| 71 | Afamelanotide | Scenesse |  |  | X |  |
| 72 | Pasireotide | Signifor | X |  |  |  |
| 73 | Hydroxycarbamide | Siklos | X |  |  |  |
| 74 | Bedaquiline fumarate | Sirturo |  |  | X |  |
| 75 | Eculizumab | Soliris | X |  |  |  |
| 76 | Edotreotide | SomaKit Toc |  |  | X |  |
| 77 | Nusinersen sodium | Spinraza |  |  | X |  |
| 78 | Asfotase alfa | Strensiq |  |  | X |  |
| 79 | Autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with retroviral vector that encodes for the human adenosine deaminase (ADA) cDNA sequence from human haematopoietic stem/progenitor (CD34+) cells | Strimvelis |  |  |  | X |
| 80 | Siltuximab | Sylvant | X |  |  |  |
| 81 | Nilotinib | Tasigna | X |  |  |  |
| 82 | Thiotepa | Tepadina | X |  |  |  |
| 83 | Thalidomide | Thalidomide Celgene |  |  | X |  |
| 84 | Tobramycin | Tobi Podhaler | X |  |  |  |
| 85 | Temsirolimus | Torisel | X |  |  |  |
| 86 | Ataluren | Translarna |  |  | X |  |
| 87 | Venetoclax | Venclyxto |  |  | X |  |
| 88 | Azacitidine | Vidaza | X |  |  |  |
| 89 | Recombinant human n-acetylgalactosamine-6-sulfatase (rhgalns) | Vimizim |  |  | X |  |
| 90 | Ambrisentan | Volibris | X |  |  |  |
| 91 | Everolimus | Votubia | X |  |  |  |
| 92 | Velaglucerase alfa | Vpriv | X |  |  |  |
| 93 | Tafamidis | Vyndaqel | X |  |  |  |
| 94 | Pitolisant | Wakix |  |  | X |  |
| 95 | 6-Mercaptopurine monohydrate | Xaluprine |  |  | X |  |
| 96 | Telotristat etiprate | Xermelo |  |  | X |  |
| 97 | Trabectedin | Yondelis | X |  |  |  |
| 98 | Allogeneic T cells genetically modified with a retroviral vector encoding for a truncated form of the human low affinity nerve growth factor receptor (LNGFR) and the herpes simplex I virus thymidine kinase (HSV-TK Mut2) | Zalmoxis |  |  |  | X |
| 99 | Miglustat | Zavesca | X |  |  |  |
| 100 | Niraparib tosylate monohydrate | Zejula |  |  |  | X |
| Total |  |  | 51 | 3 | 32 | 14 |

CN: Código Nacional; EMA: Agencia Europea de Medicamentos.

aConsta como comercializado en la Agencia Española de Medicamentos y Productos Sanitarios, pero no se ha encontrado fecha de comercialización en Bot PLUS. Se incluyó como «financiado», pero no se utilizó para el análisis de tiempos.

Fuente: análisis de los autores, basado en la información pública disponible sobre los medicamentos huérfanos autorizados por la EMA10, sobre los autorizados por la EMA con designación huérfana vigente11, sobre la fecha de asignación del CN12 y sobre la fecha de comercialización efectiva en España13.