| **DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION** | **DRUG TYPE AND USE** | **LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION** | **PBAC OUTCOME** |
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| **AMINO ACID FORMULA WITH VITAMINS AND MINERALS WITHOUT PHENYLALANINE AND TYROSINE**  30 x 36 g sachets,  TYR Anamix Junior®  Nutricia Australia Pty Ltd  Change to listing  (Minor submission) | Medicinal food | To advise of an upgrade in the nutritional formula, flavour and packaging change from 29 g sachets to 36 g sachets. | The PBAC deferred its recommendation until further advice could be provided by the Nutritional Products Working Party (NPWP) on this submission in view of the additional information received from the sponsor. |
| Sponsor Comment: | The sponsor had no comment. |
| **AMINO ACID FORMULA WITH VITAMINS AND MINERALS WITHOUT VALINE, LEUCINE AND ISOLEUCINE**  30 x 36 g sachets,  MSUD Anamix® Junior  Nutricia Australia Pty Ltd  Change to listing  (Minor submission) | Medicinal food | To advise of an upgrade in the nutritional formula, flavour and packaging change from 29 g sachets to 36 g sachets. | The PBAC deferred its recommendation until further advice could be provided by the Nutritional Products Working Party (NPWP) on this submission in view of the additional information received from the sponsor. |
| Sponsor Comment: | The sponsor had no comment. |
| **AMINO ACIDS FORMULA WITH VITAMINS AND MINERALS WITHOUT PHENYLALANINE**  30 x 36 g sachets,  PKU Anamix® Junior    Nutricia Australia Pty Ltd  Change to listing  (Minor submission) | Medicinal food | To advise of an upgrade in the nutritional formula, flavour and packaging change from 29 g sachets to 36 g sachets. | The PBAC deferred its recommendation until further advice could be provided by the Nutritional Products Working Party (NPWP) on this submission in view of the additional information received from the sponsor. |
| Sponsor Comment: | The sponsor had no comment. |
| **AMINO ACIDS FORMULA WITH VITAMINS AND MINERALS WITHOUT PHENYLALANINE**  30 x 130 mL pouches  30 x 174 mL pouches  PKU Air  Vitaflo Australia Pty Ltd  New listing  (Minor submission) | Medicinal food | Restricted benefit for phenylketonuria. | The PBAC deferred its recommendation until further advice could be provided by the Nutritional Products Working Party (NPWP) on this submission in view of the additional information received from the sponsor. |
| Sponsor Comment: | The sponsor had no comment. |
| **NITISINONE**  2 mg capsule, 60  5 mg capsule, 60  10 mg capsule, 60  Orfadin®  A.Menarini Australia Pty Ltd  New Listing  (Major submission) | Hereditary tyrosinaemia type 1 | Section 100 (Highly Specialised Drugs) listing for the treatment of hereditary tyrosinaemia type 1 (HT-1). | The PBAC deferred making a recommendation regarding the proposed Authority Required listing for nitisinone under Section 100 for treatment of HT-1 due to a lack of clarity regarding current and future screening practices for detecting HT-1 and the subsequent impact on the significant survival advantage and adverse effects observed during treatment with nitisinone.  The PBAC considered that sensitive and specific succinylacetone neonatal screening program would increase the nitisinone treatment costs, as patients commence treatment earlier but would also improve patient outcomes. This would impact on the cost-effectiveness of treatment.  On the basis of one single-arm study presented by the submission, for every 100 patients treated with nitisinone, approximately 94 are alive after 2 years of treatment regardless of age of commencing nitisinone treatment.  On the basis of a second single-arm study presented by the submission, for every 100 patients not treated with nitisinone:  • Approximately 29 are alive after 2 years if clinical symptoms of HT-1 developed before 2 months of age;  • Approximately 74 are alive after 2 years if clinical symptoms of HT-1 developed between 2 and 6 months of age; and  • Approximately 96 are alive after 2 years if clinical symptoms of HT-1 developed after 6 months of age.  The PBAC noted that adverse events that may be associated with treatment with nitisinone for HT-1 include eye disorders, haematological events and developmental and cognitive disorders. The relationship of these events to nitisinone treatment is unknown.  The PBAC recommended a stakeholder meeting be held between the sponsor, clinicians from applicable professional bodies, consumer representatives and PBAC members. The aim of this meeting would be to provide clarity about the clinical effectiveness of nitisinone for HT-1 with respect to current and future screening programs. The meeting would also provide an opportunity to consider the need for a progressive neurocognitive monitoring and assessment program, as well as an appropriate restriction arrangement. |
| Sponsor Comment: | A.Menarini Australia Pty Ltd is committed to partnering with the PBAC to make nitisinone available on the PBS for patients with tyrosinaemia type 1. |
| **PONATINIB**  15 mg tablet, 60  45 mg tablet, 30  Iclusig®  Specialised Therapeutics Australia Pty Ltd  New Listing  (Major submission) | Leukaemia | Authority Required listing for ponatinib for treatment of adult patients:  - with chronic myeloid leukaemia (CML) who are resistant or intolerant to dasatinib or nilotinib, or who have the T315I mutation; or  - with Philadelphia chromosome positive (Ph+) acute lymphoblastic leukaemia (ALL) who are resistant or intolerant to dasatinib, or who have the T315I mutation. | The PBAC deferred the submission to request a revision of the restrictions to ensure ponatinib is used in patients who can benefit most and to request a significant price reduction of ponatinib to account for the significant costs associated with the care of patients with vascular occlusive events induced by ponatinib.  Based on the data presented in the submission and the data reviewed in previous applications for imatinib, dasatinib and nilotinib, the PBAC considered that ponatinib has similar effectiveness compared with dasatinib or nilotinib in a number of clinical scenarios noting that ponatinib is active at inducing transient responses in Ph + ALL with the T315I mutation.  Based on the trial data presented in the submission and FDA information, the PBAC considered that ponatinib has an inferior toxicity profile to imatinib, dasatinib and nilotinib, especially with regards serious vascular occlusive events. |
| Sponsor Comment: | Given the urgent life saving need for an active therapy to treat patients with CML who are refractory to previous TKI therapies, Specialised Therapeutics looks forward to meeting and working with the PBAC to find a rapid and commercially feasible solution to achieve equitable access to Iclusig for all Australians. |
| **TRASTUZUMAB EMTANSINE**  100 mg injection, 1 x 100 mg vial, 160 mg injection, 1 x 160 mg vial;  Kadcyla®,  Roche Products Pty Ltd  New Listing  (Major submission) | HER2 positive metastatic breast cancer | Section 100 listing for trastuzumab emtansine (T-DM1) for treatment of a patient with HER2+ metastatic breast cancer who has received prior treatment with trastuzumab and a taxane and whose disease has progressed despite treatment with trastuzumab for metastatic disease. | The PBAC decided to defer its decision on trastuzumab emtansine (T-DM1), noting uncertainties in the economic model by omitting post-progression costs, the nature of the treatment effect and the place of T-DM1 once pertuzumab would be available. The PBAC noted the clinical need for T-DM1, however emphasised the limitations in the applicability of the evidence in the key trial given that patients had progressed on trastuzumab + taxane rather than pertuzumab + trastuzumab + taxane as is most likely to occur in clinical practice.  Subsequent to the meeting, the sponsor presented a pricing proposal for trastuzumab emtansine which was considered by the PBAC out of session. The sponsor’s proposal reduced the ICER for trastuzumab emtansine versus lapatinib + capecitabine.  The PBAC recommended the listing of trastuzumab emtansine under Section 100 (Efficient Funding of Chemotherapy Drugs Program) for treatment of a patient with HER2 positive metastatic breast cancer who has received prior treatment with trastuzumab and a taxane and whose disease has progressed despite treatment with trastuzumab for metastatic disease. |
| Sponsor Comment: | The sponsor is working with the PBAC to achieve the earliest possible PBS listing for trastuzumab emtansine (KADCYLA), addressing the high clinical need for effective treatments for Australians with metastatic breast cancer. |