European Medicines Agency
Evaluation of Medicines for Human Use

London, September 2006
Doc. Ref.: EMEA/381922/2006

ASSESSMENT OF THE PAEDIATRIC NEEDS IMMUNOLOGY

DISCLAIMER

The Paediatric Working Party (PEG) is working to identify the needs in the different therapeutic areas where there should be research and development of medicinal products for children, either old (i.e. off patent) or new ones (including those under development). Products on the list are not in any order of priority.

The lists should not be viewed as a prescription tool nor as recommendations for treatment. Accuracy of data including in particular authorised doses cannot be guaranteed.

Information on existing marketing authorisations is very limited and therefore information under "authorised" includes the indication in broad term (only related to chemotherapy), the lower age group authorised in at least one Member State, the authorised dose(s) (if authorised for use in patients less than 18 years of age) and formulation(s) in at least in one Member State.

Please refer to the EMEA/PEG procedure for identifying the paediatric needs for further information.

Comments from third parties are expected especially to complete and or update the list as necessary.

AGREED BY PAEDIATRIC WORKING PARTY (PEG)	November 2005
ADOPTION BY CHMP FOR RELEASE FOR CONSULTATION	December 2005
END OF CONSULTATION (DEADLINE FOR COMMENTS)	June 2006
AGREED BY PAEDIATRIC WORKING PARTY (PEG)	06 October 2006
ADOPTION BY CHMP FOR FINAL RELEASE	19 October 2006

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If not stated separately, the need for availability in all Member States of the Community applies to all medicinal products included in this list.

CYTOKINE INHIBITORS	
CICLOSPORIN	
Authorised indication	Solid organ transplantation, bone marrow transplantation, nephrotic syndrome, endogenous uveitis, minimal change nephropathy, focal and segmental glomerulosclerosis, membranous glomerulonephritis, rheumatoid arthritis, psoriasis, severe atopic dermatitis
Authorised age group	Children (age group unspecified)
Authorised dose	In transplantation as adults (therapeutic drug monitoring), 5mg/kg/d for nephrotic syndrome (starting dose)
Authorised formulation	50 mg/ml concentrate for dilution for infusion 10, 25, 50 and 100 mg capsules and 100 mg/ml oral solution
Needs	Need for PK data in children < 1 year. 10 mg capsules should be available in all member sates Age appropriate formulation Extension of indication in aplastic anemia; hemophagocytotic lymphohistiocytosis
TACROLIMUS	
Authorised indication	Prophylaxis of transplant rejection in liver, kidney or heart allograft recipients and treatment of allograft rejection resistant to treatment with other immunosuppressive medicinal products.
Authorised age group	> 2 years (liver and kidney)C
Authorised dose	Liver and Kidney: initial oral dose of 0.30 mg/kg per day in two divided doses. Heart: Without antibody induction: Initiation i.v. starting dose 0.03-0.05 mg/kg/day, then p.o. starting dose of 0.30 mg/kg/day. With antibody induction: Initial oral dose 0.10-0.30 mg/kg/day. Maintenance doses in children are often 1.5-2 times adult dose
Authorised formulation	0.5, 1 mg and 5 mg capsules; 5mg/ml concentrate for dilution for infusion
Needs	Need for 0.25 mg capsule, oral suspension, PK, efficacy and safety < 2 years Based on the mechanism of action, to define the potential effect of the product in various immunology indications (including Bone Marrow Transplantation) and where appropriate study its efficacy and safety
ANTIMETABOLITES	
AZATHIOPRINE	
Authorised indication	Organ transplantation, autoimmune diseases
Authorised age group	Children (age group unspecified)
Authorised dose	Same as adults; starting dose 2-5mg/kg per day for transplants; 1-2.5mg/kg per day for other indications
Authorised formulation	25 and 50 mg scored tablets (*France*), powder for injection, i.v.
Needs	Define lower age limit based on available data on efficacy and safety Oral liquid formulation Based on the mechanism of action, to define the potential effect of the product on various immunological indications and where appropriate study its efficacy and safety
MYCOPHENOLATE MOFETIL	
Authorised indication	Prevention and treatment of rejection in kidney transplants
Authorised age group	Children > 2 years
Authorised dose	600 mg/m² BID via oral route
Authorised formulation	250 mg capsules, 500 mg tablets

	1g/5ml powder for oral suspension. 500 mg Powder for concentrate for solution for infusion (adult only)
Needs	Need for i.v. route indication, PK, efficacy and safety <2 years Based on the mechanism of action, to define the potential effect of the product on various immunology indications (including other organ transplantations and treatment of autoimmune diseases e.g. Lupus nephritis) and where appropriate study its efficacy and safety
MYCOPHENOLATE SODIUM	
Authorised indication	Prevention and treatment of rejection in kidney transplants
Authorised age group	> 18 years
Authorised dose	-
Authorised formulation	-
Needs	Extension of indication in all age groups (efficacy, long term safety data and dose) Age appropriate formulation
SELECTIVE IMMUNOSUPPRESSANTS	
SIROLIMUS	
Authorised indication	Kidney transplant
Authorised age group	> 18 years
Authorised dose	-
Authorised formulation	1 mg/ml, 2 mg/2ml, 5 mg/5ml oral solution; 1, 2, 5 mg coated tablets
Needs	Extension of the indication (pharmacokinetics, efficacy, long term safety data and dose) in all age groups including newborns, based on the mechanism of action, to define the potential effect of the product on various immunology indications Age appropriate formulation
EVEROLIMUS	
Authorised indication	Kidney and heart transplant
Labelled age group	> 18 years
Labelled dose	-
Labelled formulation	0.1, 0.25, 0.5, 0.75, 1.0 mg tablets
Needs	Extension of the indication (efficacy, long term safety data and dose) in all age groups including newborns Age appropriate formulation
ANTILYMPHOCYTE ANTIBODIES	
MUROMONAB-CD3	
Authorised indication	Acute rejection after de novo kidney, heart, liver transplants
Authorised age group	Children *(Spain)*
Authorised dose	-
Authorised formulation	1 mg/ml injection solution
Needs	Extension of indication to Bone Marrow Transplant (BMT) and treatment of GvHD (dose, efficacy and safety) as conditioning regimen from > 1 year
BASILIXIMAB	
Authorised indication	Prevention of acute rejection after de novo allogeneic kidney transplants
Authorised age group	> 1 year
Authorised dose	Children > 35 kg: 20 mg x 2 as adults Children < 35 kg: 10 mg x 2
Authorised formulation	20 mg and 10 mg injection
Needs	Dose, efficacy and safety in liver, heart, and lung transplant, bone marrow transplantation < 1 year

DACLIZUMAB	
Authorised indication	Prevention of acute rejection after de novo allogeneic kidney transplants
Authorised age group	Children (age group unspecified)
Authorised dose	1 mg/kg every 14 days, total = 5 doses. Same as adults
Authorised formulation	Concentrate for solution for infusion.
Needs	Based on the mechanism of action to define the potential effect in other indications

RITUXIMAB	
Authorised indication	Prevention of acute rejection after de novo allogeneic kidney transplants, lymphoma
Authorised age group	> 18 years
Authorised dose	-
Authorised formulation	Solution for dilution for i.v. infusion 100, 500 mg.
Needs	Based on the mechanism of action to define the potential effect in other indications, such as post-transplant lymphoproliferative disease, severe autoimmune haemolytic anemia, refractory ITP
POLYCLONAL ANTIBODIES	
ATG THYMOGLOBULINE	
Needs	Development in children of all age groups for indication bone marrow transplantation, kidney and renal transplantation, severe autoimmune diseases and aplastic anemia
ALG LYMPHOGLOBULINE	
Needs	Development in children of all age groups for indication bone marrow transplantation, kidney and renal transplantation and aplastic anemia
ALKYLATING AGENTS	
CYCLOPHOSPHAMIDE	
Authorised indication	Immunosuppressant for rare autoimmune disorders such as Wegener's granulomatosis, Goodpasture syndrome etc., corticoid dependent idiopathic nephritic syndrome
Authorised age group	Children (age group unspecified)
Authorised dose	3 mg/kg per day
Authorised formulation	50, 200, 500, 1000, 2000 mg coated tablet injectable form 100, 500, 1000 mg
Needs	Need for age appropriate formulations, such as 5 mg, 10 mg, 15 mg and 30 mg capsules and liquid formulation; harmonize authorisation and define lower age limits in children across Member States
CORTICOSTEROIDS	
PREDNISOLONE	
Authorised indication	Transplantation and immunological disorders
Authorised age group	No age limit
Authorised dose	The dose should be adapted according to disease and body weight
Authorised formulation	5 mg and 20 mg tablets, 5 mg scored tablets (reserved for adults and children > 6 years), 1 mg/ml oral solution reserved for infants and young children Coated tablets (1, 2, 2.5, 5, 6, 20 and 50 mg), soluble tablets, oral liquid formulations *(France)*, soluble tablets *(United Kingdom)*
Needs	Age appropriate oral formulation to be made available in all MSs
PREDNISONE	
Authorised indication	Transplantation and immunological disorders
Authorised age group	Children (age group unspecified)
Authorised dose	The dose should be adjusted according to disease and body weight

Authorised formulation	1 mg and 5 mg, 20 mg, 40 mg tablets
Needs	Age appropriate oral formulation to be made available in all MSs. Prednisolone solution has a very bitter taste, hard to swallow for children.
HYDROCORTISONE	
Authorised indication	Glucocorticoid treatment in adrenal insufficiency (*France*), aphthous ulcers as occurring in colitis ulcerosa (*United Kingdom*)
Authorised age group	Children (age group unspecified)
Authorised dose	12 à 20 mg/m^2
Authorised formulation	Powder for injection solution, 10, 20 mg tablets; oral pellets (*United Kingdom*)
Needs	Age appropriate liquid formulation
FLUTICASONE	
Authorised indication	Eczema/dermatitis
Authorised age group	> 1 year
Authorised dose	2x daily, < 4 weeks
Authorised formulation	Cream
Needs	Extension of indication < 1 year

UNMET MEDICAL NEEDS	
Indications:	Various immunology indications
Safety	Studies on the impact on immune system in maturation and risk of secondary tumours