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# Timely access to therapies for severe diseases with unmet medical need: a proposal for the European Countries

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# Timely access to therapies for severe diseases with unmet medical need.

## 1. Introduction

In many European Countries patients have to wait a long period of time before the medicines, approved by the European Commission on the basis of the EMA/CHMP positive opinion, are really available in a democratic way.

Many efforts have been done to improve the situation and in some particular cases national legislation, like the French ATU and the Italian 648 Law, have addressed this issue although with a certain degree of variability among treatments. However for many severe diseases and in many Member States the situation is far from being satisfactory.

The European legislation has provided many tools to favor the early availability of therapies, when a severe disease is concerned and there is an unmet medical need.

In fact the Orphan Medicinal Product status (according to Regulation 141/2000), the conditional approval (according to Regulation 507/2006) and the accelerated procedure (according to Regulation 726/2004) have been conceived to favor the early availability of therapy for severe diseases with an unmet medical need. The recent “Reflection paper on a proposal to enhance early dialogue to facilitate accelerated assessment of priority medicines (PRIME)” expresses the same objective.

However in many cases these admirable intentions are frustrated by the time requested by the national procedures for the reimbursement, which are mandatory for a real democratic availability of the therapy to the patients.

One interesting exception is the German Act on Reform of the Market for Medical Products (AMNOG). According to this law, right after the European Marketing Authorization, a medicine can be reimbursed at a price defined by the owner of the MA. At the same time a procedure for the negotiation starts. At the end of the negotiation the price will be modified accordingly.

However this German procedure cannot be proposed for all the European Countries, because most of them have not the resources to bear the connected economic impact or have different political agenda with lower willingness to invest on pharmaceuticals / healthcare.

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## 2. Patient need

The priority for patients with a severe disease, life threatening or strongly debilitating, is the availability of the best therapy as soon as the positive benefit / risk ratio has been established. This is particularly true if the new therapy addresses an unmet medical need or has demonstrated a significant clinical benefit compared with the available treatments.

## 3. Rational for a new proposal for the real timely availability for *"priority treatments"*

Presently the evaluation of the severity of the disease and the unmet medical need, at European level, is already done in the following cases:

- For Orphan Medicinal Products by COMP, according to Regulation 141/2000
- For the extension of indication by CHMP (on the basis of a request by the applicant) (Article 14(11) of the Regulation 726/2004)
- For the Conditional MA by CHMP, according to Regulation 507/2006
- For the Accelerated procedure by SAWG/CHMP, according to Regulation 726/2004

In the future also the "PRIME" procedure will include this evaluation according to the *"Reflection paper on a proposal to enhance early dialogue to facilitate accelerated assessment of priority medicines (PRIME)"*

In conclusion in these cases the centralized procedure for the marketing authorization includes an assessment, made by CHMP or COMP, regarding the severity of the disease and the unmet medical need or the significant benefit.

It is worth reminding that, in case of Orphan Medicinal Products, Regulation 847/2000 article 3 clarifies that "significant benefit" means a "***clinically relevant advantage or a major contribution to patient care" .***



*In this proposal "recent" and "recently" mean no more than 6 months before the Marketing Authorization

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## 4. New Proposal : additional administrative "Special Timely Procedure" for the reimbursement of therapies for severe diseases having an unmet medical need or a significant benefit in comparison with the existing therapies.

For therapies for severe diseases and an unmet medical need or a "significant benefit" as evaluated recently* by CHMP or by COMP, Member States, on a voluntary basis, trigger an administrative mechanism of immediate reimbursement and so of immediate availability for all the patients in need. Because also the compatibility with the economic resources has to be respected, a special national procedure has to be studied in the details, taking into account sustainability.

With the aim of making this administrative national procedures sustainable, the following limitations are suggested:

a) The national administrative ***"special timely procedure"*** will be used just for therapies for **severe diseases**, which means life threatening or chronically debilitating conditions, according to a recent* CHMP or COMP evaluation. The definition of "severe diseases" is already defined in the European Regulation for OMPs (Regulation 141/2000, article 3).

b) The national administrative ***"special timely procedure"*** will be used just for therapies recently* recognized by the CHMP or by COMP to address an **unmet medical need or to have a significant benefit** in comparison with the current therapies for the same condition (as in the European Regulation 847/2000, article 3).

c) The national administrative ***"special timely procedure"*** will be restricted to the following cases:

   a. Orphan Medicinal Products according to Regulation 141/2000

   b. Extension of indication with significant benefit according to Article 14(11) of Regulation 726/2004

   c. Conditional MA according to Regulation 507/2006

   d. MA approved through the accelerated procedure according to Regulation 726/2004

d) The national administrative **"*special timely procedure"*** will be used just for Marketing Authorization or extension of indication following a positive opinion by CHMP by consensus.

e) The national administrative ***"special timely procedure"*** allows the **immediate reimbursement** by the NHS: no additional assessment by the NHS is necessary

f) The **initial price** is decided by the sponsor, and it is equal to the lowest price for the same medicine available in the European Union. This price could be immediately aligned in case other lower prices would be agreed, at the end of the usual procedure for price and reimbursement, in Countries sharing this special national timely procedure

g) Possible **100% payback** by the sponsor, when the usual National procedure of negotiation will be finished, equal to the difference between the used free price and the price established at the end of the procedure.

h) A national "**Register**" for the new treatment, if requested by the National Authority, could be placed effective from the initial drug availability

i) A **maximum turnover** (ceiling), in the first 12 months could be fixed: for example 0.5% of the total national expenditure for medicines reimbursed by the NHS. In case the expected expenditure is higher, the sponsor has two alternatives:

   a. Renounce this national timely procedure

   b. Supply the medicine as free of charge after the fixed ceiling during the first 12 months.

j) **Failure** of the usual P&R negotiation: a failure of the usual national negotiation is quite unlikely in case of therapies for unmet need or with additional clinical benefit for severe diseases. In any case, if a failure occurs, the applicant will be obliged to payback a certain percentage of the turnover (for example 20%) and the medicine will be classified in class C (this outcome is valid for Italy: for other Countries a specific clause has to be studied, Country by Country, depending on the National legislation).

k) This administrative ***"special timely procedure"*** does not substitute but is in addition to the usual national procedure for P&R negotiation. It has the objective of reducing the time between the European Marketing Authorization and the real availability in the Country for therapies with an additional significant benefit and for severe diseases.

l) This administrative ***"special timely procedure"*** does not substitute but is in addition and could be synergetic to the procedure under evaluation by EMA and EUnetHTA for a "first European HTA at the time of the MA". It has the limited objective to reduce at a minimum the gap in time between the MA and the real availability of the medicine in many Member States.

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