



## Priority Medicines for Europe and the World Update 2013 report

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Brussels  
July 9<sup>th</sup> 2013



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World Health  
Organization

2004

Leufkens 2017

2014

## Challenging innovation scenarios in pharma

| Scenario<br>Criterion                   | Disease<br>mongering | <b>Orphan<br/>diseases</b> | Drug<br>rediscovery |
|-----------------------------------------|----------------------|----------------------------|---------------------|
| Unmet medical need,<br>patient benefit  | No/Debatable         | <b>Yes</b>                 | Yes                 |
| Science is there                        | Yes                  | <b>Strong<br/>increase</b> | Variable            |
| Value creation,<br>viable business case | Yes                  | <b>Variable</b>            | Variable            |
| Regulatory<br>constraints               | Mostly not           | <b>Modest</b>              | Variable            |

# Determinants for successful marketing authorisation of orphan medicinal products in the EU

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- 2000-2010 >850 orphan designations.
- 1 out of 4 a 'biological'.
- 114 'applications'; 64% approved.
- Of approved products 71.2% on the basis of RCT; 83.1% with clinically relevant effect on primary endpoint.

Drug Discov Today 2012; 17:352-358.