Value assessment and pricing frameworks for rare disease treatments: new approaches from the literature

Palaska C*. Hutchings A.

Dolom, London, United Kingdom.

*Correspondence: christina.palaska@dolom.com

Background and objectives

- It has been 15 years since the introduction of orphan medicinal product (OMP) legislation in the EU. Until 2014, one hundred and twelve orphan drugs had been approved in Europe.
- Orphan drugs represent a particular challenge to pharma due to the large unmet patient need, paucity of disease information and high development costs. As of yet, there is no consensus on the most appropriate framework to assess the value of OMPs and to determine the price level at which they should be funded.

Methods

- A systematic literature review was conducted using Medline and EMBASE databases and conference proceedings with an additional hand search for the period 2003–2014 without geographic restriction.
- The search sought to identify papers that proposed specific frameworks for assessing the value of rare disease treatments or to determining the price of orphan drugs. Eleven papers were included. Clinical or economic studies of specific rare diseases and their treatments were excluded.
- The proposed frameworks were considered in respect to:
  - Scope and purpose
    - Elements of value included.

Results

- The literature review identified 1,334 papers including publications, conference papers and a hand search. Of these, eleven papers were selected which included information on new specific frameworks for assessing and/or pricing orphan drug treatments. Figure 1 depicts a PRISMA flow diagram for the literature review.
- The nine frameworks are summarized in Table 1. Three of the frameworks were country-specific, three European focused, and three international or region specific. All were published since 2012.

Scope and purpose

- There is no consensus in the literature on what the scope of a rare disease assessment framework should encompass. Broadly, two key areas are considered in the frameworks: assessment of product value and mechanisms for determining price or funding place.

Structure and methodology

- The most commonly proposed methodology for the valuation framework was a variant of MCDA (Table 1). Authors highlighted the feasibility of such an approach to include a broader range of relevant factors that are pertinent to rare diseases.

Table 1: Summary of proposed assessment frameworks for rare disease treatments

<table>
<thead>
<tr>
<th>Framework</th>
<th>Year</th>
<th>Type</th>
<th>Methodology</th>
<th>Elements considered</th>
<th>Weighting</th>
<th>Value calculation</th>
<th>Price/funding decision</th>
</tr>
</thead>
<tbody>
<tr>
<td>Winquist et al.</td>
<td>2013</td>
<td>Europe</td>
<td>MCDA-type framework</td>
<td>Disease characteristics, treatment characteristics, economic and healthcare system aspects</td>
<td>Not specified</td>
<td>Value is derived from the sum of weighted criteria scores</td>
<td>Determine funding status</td>
</tr>
<tr>
<td>NICE</td>
<td>2012</td>
<td>Canada</td>
<td>Model-based approach</td>
<td>Disease characteristics, treatment characteristics, economic and healthcare system aspects</td>
<td>Not specified</td>
<td>Value is derived from the sum of weighted criteria scores</td>
<td>Determine funding status</td>
</tr>
<tr>
<td>Palaska et al.</td>
<td>2014</td>
<td>Europe</td>
<td>MCDA-type framework</td>
<td>Disease characteristics, treatment characteristics, economic and healthcare system aspects</td>
<td>Not specified</td>
<td>Value is derived from the sum of weighted criteria scores</td>
<td>Determine funding status</td>
</tr>
<tr>
<td>Fedyaeva et al.</td>
<td>2014</td>
<td>Russia</td>
<td>MCDA-type framework</td>
<td>Disease characteristics, treatment characteristics, economic and healthcare system aspects</td>
<td>Not specified</td>
<td>Value is derived from the sum of weighted criteria scores</td>
<td>Determine funding status</td>
</tr>
<tr>
<td>Palaska C* et al.</td>
<td>2015</td>
<td>Europe</td>
<td>MCDA-type framework</td>
<td>Disease characteristics, treatment characteristics, economic and healthcare system aspects</td>
<td>Not specified</td>
<td>Value is derived from the sum of weighted criteria scores</td>
<td>Determine funding status</td>
</tr>
</tbody>
</table>

Conclusions

- There is a need for a commonly accepted framework for making pricing and funding decisions for orphan drugs.
- Currently, there is no consensus in the literature on the structure of such a framework nor the core elements for consideration.
- MCDA-type frameworks are most frequently proposed, and there is increasing convergence on the core value elements.
- The issue that requires most consideration and further research is the process by which value is translated to price.
- There is considerable variation in methodology used by authors when describing rare disease frameworks, which complicates the task of reaching consensus.

Presented at EUROR2015, Milan.