Regulatory perspective

Rare Cancers Europe Webinar on “Rare Cancers in All Policies”

Presented by Antonella Baron 28 September 2021
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Disclaimer

The presenter does not have any conflict of interest

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New medicines to treat a rare cancer: where to start?

- Data in my spreadsheet?
- Natural disease history?
- Standard of care?
- Single arm trial?
- Funding?
- Repurposing?
- Publications?
- Orphan or ultraorphan?
- Adults? Children?
- Similar vs specific features?
- Funding?
- Repurposing?
- Standard of care?
- Natural disease history?
- Single arm trial?
opportunities for dialogue with EMA

Medicine development

Product discussions

Workshop with study groups, patients, companies

Small populations: different regulatory frameworks built for purpose

<table>
<thead>
<tr>
<th>Category</th>
<th>Frameworks</th>
</tr>
</thead>
<tbody>
<tr>
<td>Orphan conditions (valid for products that received an orphan designation)</td>
<td>Orphan designation, Protocol Assistance, PRIME</td>
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<tr>
<td>Paediatric conditions (some are rare, need dedicated research)</td>
<td>Scientific advice, PIP, potentially PUMA, PRIME</td>
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<tr>
<td>Subsets of diseases (e.g. biomarker driven indications)</td>
<td>ITF, Scientific advice, PRIME</td>
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<tr>
<td>Personalised medicine (innovative trials, out of the box proposals for regulators)</td>
<td>ITF, Scientific advice, PRIME</td>
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# Medicines recommended for approval in the last 3 years

<table>
<thead>
<tr>
<th></th>
<th>2018</th>
<th>2019</th>
<th>2020</th>
</tr>
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<tbody>
<tr>
<td>Medicines recommended for approval</td>
<td>84</td>
<td>66</td>
<td>97</td>
</tr>
<tr>
<td>Total Solid and Blood Cancer</td>
<td>24</td>
<td>13</td>
<td>32</td>
</tr>
<tr>
<td>Rare Solid Cancer + Rare Blood Cancer</td>
<td>6</td>
<td>2</td>
<td>8</td>
</tr>
<tr>
<td>Rare disease (orphan designation at the time of CHMP opinion)</td>
<td>21</td>
<td>7</td>
<td>22</td>
</tr>
<tr>
<td>Conditional marketing authorisation</td>
<td>3</td>
<td>0</td>
<td>3</td>
</tr>
<tr>
<td>Exceptional circumstances</td>
<td>0</td>
<td>1</td>
<td>2</td>
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</tbody>
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**Conditional marketing authorization:**
- unmet medical needs
- less complete clinical data than normally required
- specific post-authorisation obligations

**Exceptional circumstances:**
- comprehensive data cannot be obtained (e.g. only very few patients with the disease)
- specific post-authorisation obligations and monitoring
Present and Future Challenge

• “Clinical research in cancer is challenging and especially so for the many rare and paediatric cancers. There is an opportunity for the EU to optimise the research effort by ensuring access to clinical trials and facilitating participation in trials when considered the best option. For this, we need to leverage the resources for conducting high quality clinical research in Europe.

• “Rare cancers will benefit from facilitating access to clinical trials that would otherwise take prohibitively long time to conduct..................need to encourage and support collaborative clinical trials leveraging collaboration between academia and network scientists to address rapidly emerging regulatory science research questions as well as treatment optimisation.”

• “the challenge is in creating and supporting the right infrastructure across Europe for systematically collecting, federating, and sharing of key data from different sources”

Emer Cooke- Executive Director EMA
Thank you for your attention

Further information

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