The regulatory and reimbursement approval process, and evidence requirement at the national level for ODs is same as for any other pharmaceutical product. The role of national organization in reviewing ODs is provided in Table 1.

Table 1: Overview of roles of national organization in reviewing ODs in Canada

<table>
<thead>
<tr>
<th>Organization</th>
<th>Role</th>
<th>Impact</th>
</tr>
</thead>
<tbody>
<tr>
<td>Health Canada</td>
<td>• Assesses product safety, efficacy and quality</td>
<td>• Market authorization</td>
</tr>
<tr>
<td>Canadian Agency for Drugs and Technology in Health (CADTH)</td>
<td>• Coordinates the Common Drug Review (CDR)</td>
<td>• National coverage reimbursement and restrictions</td>
</tr>
<tr>
<td>Canadian Institutes for Health Research (CIHR)</td>
<td>• Prepares evidence-based drug formulary listing recommendations to the publicly funded provincial drug plans</td>
<td>• National price determination</td>
</tr>
</tbody>
</table>

Methods

Non-systematic PubMed search, Health Canada, the Canadian Agency for Drug and Technology in Health (CADTH), Common Drug Review (CDR), Canadian Organization of Rare Diseases (CORD) and different provincial Ministries of Health websites.

Results

In Ontario, there is no difference in funding between orphan and non orphan drugs and all are covered from the app. $4 billion fund for prescription drug products. For most orphan drugs, prescribing restriction exists e.g. prescribing by specialist at the Centre of reference according to clinical guidelines and reimbursement approval on a named patient basis.

In Alberta, payers split up funding for ODs into a separate budget which allows them to track spending on these drugs. Manufacturers can expect similar prescribing restrictions in Alberta that to Ontario. From the Alberta formulary, it is understandable that the output of one of the many variables used by the Executive Officer to make a funding decision by identifying those groups of patients most likely to achieve potential benefit.

In Quebec, the model output help to identify groups of patients which are most likely to achieve potential benefits of the drug treatment. The output model is based on the work of an expert committee of a panel of specialists who make decisions on treatment guidelines, criteria for coverage and monitor response to therapy.

Conclusions

In the absence of a national orphan drug policy, patients suffering from rare diseases face challenges in obtaining access to ODs in Canada. However, currently opportunities exist for manufacturers to provide access to those ODs which have a genetic cause and low prevalence in Canada.


Pricing and reimbursement of orphan drugs in Canada

The main objectives of this poster were to define ODs regulations and market access barriers faced by manufacturers in Canada. Being a socialized healthcare system and heavily focused on cost containment, market access for ODs in Canada is highly regulated. In the absence of a national orphan drug policy, patients suffering from rare diseases face challenges in obtaining access to ODs in Canada. However, currently opportunities exist for manufacturers to provide access to those ODs which have a genetic cause and low prevalence in Canada.

1. Kumar J, Bachman EM, Heron Evidence Development LLC, SOMERSE, NJ USA

1.2 Heron Evidence Development LLC, SOMERSE, NJ USA

http://www.heronhealth.com