PATIENT-REPORTED OUTCOMES (PRO) IN POST-PROGRESSION ONCOLOGY: IMPLICATIONS IN HEALTH TECHNOLOGY ASSESSMENTS & PAYER DECISION MAKING

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BACKGROUND

In addition to data regarding safety and efficacy, patient reported outcomes (PROs) are an accepted and often actively solicited source of evidence used by health authorities and payers in evaluating and approving pharmaceutical interventions, and are increasingly used across disease areas, including oncology. The role of PROs in drug approval is particularly important for products developed to treat chronic, disabling conditions where the intention is not necessarily to cure, but to ameliorate symptoms, facilitate function, or improve quality of life.1,2 However, in oncology it is not common to continue to collect PRO data after progression free survival (PFS) has been reached. Therefore, we conducted a study to understand how payers perceived the value of PRO data after progression.

OBJECTIVE

To assess the impact of PRO data collected after clinical progression (i.e., post-progression) on payer decision making in oncology.

METHODS

One-on-one interviews were conducted with 16 payers and payer advisors from 14 countries in 2014. An online assessment was conducted December 8, 2014, to March 4, 2015, with 20 completed surveys (China, France, Germany, Spain, Taiwan, the UK) and 7 partially completed surveys (Australia, South Korea, the US) by payers from the RTI Health Solutions Global Payer Advisory Panel.

The profiles of US and ex-US payers and payer advisors interviewed are listed in Tables 1 and 2, respectively.

RESULTS

In response to the question of whether registrant data were important in payer decision making for oncology, 70% of respondents said yes. Post-progression data may support further use of the therapy, even if the tumor is still progressing. Respondents indicated that PRO data may help differentiate treatment post-progression and could impact decision making, particularly in the future.

Post-progression PRO data could be useful if two drugs and one has inexpedient PRO data more compared to second and/or was less severe the drug with PRO data. If marginal endpoint improvement and PRO data then more likely to manage lightly.

Online respondents from the US indicated they were aware of examples of oncology products that received favorable decisions because of PRO data and conversely did not receive favorable reimbursement decisions because they did not include PRO data.

Online respondents generally rated post-progression PRO data as useful.

Ex-US respondents rated usefulness of post-progression PRO data for an oncology therapeutic higher than US respondents (Figure 1) and the usefulness of PROs collected post-progression varied among US respondents.

US respondents generally rated post-progression PRO data as useful

Ex-US respondents rated usefulness of post-progression PRO data for an oncology therapeutic higher than US respondents (Figure 1)

- US Pharmacy Director

Reimbursement for one on one interview and online survey indicated the collection of PRO data, as well as post-progression PRO data were important in payer decision making for oncology therapeutics.

Post-progression data may support further use of the therapy, even if the tumor is still progressing.

PRO data in oncology will increase in importance over the next 5-10 years, including PRO data measured post-progression.

What type of data (PRO or other) should be collected in the post-progression period?

- Payers generally thought all types of data queried were important to decision making with some of data rated as being of higher importance (i.e., tier 2).

Figure 2: Rating of the Value of Post-progression PRO data by type

Sweden

France

“I...very important, especially in advanced metastatic stage of cancer.”

“One of the issues in the assessment of oncology products is that there is so much focus on PFS and what is happening after that is not explored.”

“...don’t like the idea of paying for a lot for a few weeks of added survival, but if QoL is better then [we are] more compelled to support coverage.”

-US Pharmacy Director

REFERENCES


CONCLUSIONS

- Respondents from one on one interview and online survey indicated the collection of PRO data, as well as post-progression PRO data were important in payer decision making for oncology therapeutics.
- Post-progression data may support further use of the therapy, even if the tumor is still progressing.
- US respondents generally rated post-progression PRO data as useful for an oncology therapeutic higher than US respondents (Figure 1) and the usefulness of PROs collected post-progression varied among US respondents.
- The longer the post-progression survival, the more important the quality of that survival is in the aggregate assessment of the cost per QALY. Therefore, it is better to collect data which are in the early to moderate state of disease.
- “The length of post-progression survival, the more important the quality of that survival is in the aggregate assessment of the cost per QALY. Therefore, it is better to collect data which are in the early to moderate state of disease.”

Table 1: Page Profiles in the United States

<table>
<thead>
<tr>
<th>Position</th>
<th>Geographic Coverage Area</th>
<th>Covered Lives</th>
<th>Total (millions)*</th>
<th>Commercial</th>
<th>Medicare</th>
<th>Medicaid</th>
</tr>
</thead>
<tbody>
<tr>
<td>Medical Director</td>
<td>National</td>
<td>11.0</td>
<td>17%</td>
<td>43%</td>
<td>5%</td>
<td></td>
</tr>
<tr>
<td>Pharmacy Director</td>
<td>National</td>
<td>35.0</td>
<td>75%</td>
<td>15%</td>
<td>15%</td>
<td></td>
</tr>
<tr>
<td>Medical Director</td>
<td>Employer Payroll</td>
<td>0.5</td>
<td>100%</td>
<td>0%</td>
<td>0%</td>
<td></td>
</tr>
</tbody>
</table>

Table 2: Payor Advisor Profiles outside the United States

<table>
<thead>
<tr>
<th>Country</th>
<th>Payor Advisor Profile</th>
</tr>
</thead>
<tbody>
<tr>
<td>Australia</td>
<td>Health economics professor and advisor to Medical Services Advisory Committee (MSAC)</td>
</tr>
<tr>
<td>Brazil</td>
<td>Health economics professor and advisor to Health Authority de São Paulo</td>
</tr>
<tr>
<td>France</td>
<td>Health economics professor and advisor to the National Institute for \</td>
</tr>
<tr>
<td>Germany</td>
<td>Health economics professor and advisor to national health agency</td>
</tr>
<tr>
<td>Italy</td>
<td>Health economics professor and advisor to regional health agency</td>
</tr>
<tr>
<td>Korea</td>
<td>Health economics professor and advisor to Health Insurance Review and Assessment (HRA)</td>
</tr>
<tr>
<td>Netherlands</td>
<td>HTA professor and advisor to Zorginstituut Nederland (ZIN, formerly ZOZ)</td>
</tr>
<tr>
<td>Poland</td>
<td>Professor and advisor to Agency for Technology Assessment (OSTA)</td>
</tr>
<tr>
<td>Spain</td>
<td>Health economics professor and advisor to regional health authorities</td>
</tr>
<tr>
<td>Sweden</td>
<td>Health economics professor and advisor to Tandements- och Mellanlivsstyrelsens (TUM)</td>
</tr>
<tr>
<td>Taiwan</td>
<td>Health economics professor and HTB advisor</td>
</tr>
<tr>
<td>Turkey</td>
<td>Health economics professor and advisor to public and private insurance providers</td>
</tr>
<tr>
<td>United Kingdom</td>
<td>Health economics professor and advisor to the National Institute for Health Care Excellence (NICE), Scottish Medicines Consortium (SMC)</td>
</tr>
</tbody>
</table>

Figure 1: How useful are PRO data collected post-progression?

Figure 2: Rating of the Value of Post-progression PRO data by type

Sweden

France

United Kingdom

Table 1

<table>
<thead>
<tr>
<th>US (n = 14)</th>
<th>7 = Extremely useful</th>
<th>6 = Very useful</th>
<th>5 = Useful</th>
<th>4 = Slightly useful</th>
<th>3 = Not very useful</th>
<th>2 = Not at all useful</th>
<th><strong>Total</strong></th>
</tr>
</thead>
<tbody>
<tr>
<td>Ex-US (n = 7)</td>
<td>7 = Extremely useful</td>
<td>6 = Very useful</td>
<td>5 = Useful</td>
<td>4 = Slightly useful</td>
<td>3 = Not very useful</td>
<td>2 = Not at all useful</td>
<td><strong>Total</strong></td>
</tr>
<tr>
<td>All (n = 21)</td>
<td>7 = Extremely useful</td>
<td>6 = Very useful</td>
<td>5 = Useful</td>
<td>4 = Slightly useful</td>
<td>3 = Not very useful</td>
<td>2 = Not at all useful</td>
<td><strong>Total</strong></td>
</tr>
</tbody>
</table>

Rated on a scale of 1 to 7 with 1 being unimportant to 7 being very important.

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“One of the issues in the assessment of oncology products is that there is so much focus on PFS and what is happening after that is not explored.”

“...very important, especially in advanced metastatic stage of cancer.”

“...don’t like the idea of paying for a lot for a few weeks of added survival, but if QoL is better then [we are] more compelled to support coverage.”

-US Pharmacy Director

“...PRO data is valuable in the end-stage of the disease where you are trying to improve QoL...”

“One of the issues in the assessment of oncology products is that there is so much focus on PFS and what is happening after that is not explored.”

“...very important, especially in advanced metastatic stage of cancer.”

“...don’t like the idea of paying for a lot for a few weeks of added survival, but if QoL is better then [we are] more compelled to support coverage.”

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