PCN324

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

Hogue SL[1], Brogan AP[1], De Muro C[1], D'Alessio D[2], Bal V[2]

[1]RTI Health Solutions, Research Triangle Park, NC, United States; [2]Novartis Pharmaceuticals Corporation, East Hanover, NJ United States

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 being 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 post-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, the US) 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.

Table 1: Payer Profiles in the United States

Position	Geographic Coverage Area	Covered Lives			
		Total (millions)[a]	Commercial	Medicare	Medicaid
Medical Director	National	11.0	17%	43%	5%
Pharmacy Director	National	35.0	70%	15%	15%
Medical Director	Employer Payer	0.5	100%	0%	0%

[a]35% Tricare

Table 2: Payer Advisor Profiles outside the United States

Country	Payer Advisor Profile
Australia	Health economics professor and advisor to Medical Services Advisory Committee (MSAC) and Pharmaceutical Benefits Advisory Committee (PBAC)
Brazil	Clinical oncologist, professor, and advisor to private insurance providers
France	Health economics professor and advisor to Haute Autorité de Santé (HAS)
Germany	Health economics professor and member of the arbitration board for drug prices in the statutory health insurance
Italy	Health economics professor and advisor to regional health agency (Lombardy, Lazio, Local/Hospital)
Korea	Health economics professor and advisor to Health Insurance Review and Assessment (HIRA)
Netherlands	HTA professor and advisor to Zorginstituut Nederland (ZINL, formerly CVZ)
Poland	Professor and advisor to Agencja Oceny Technologii Medycznych (AOTM)
Spain	Health economics professor and advisor to regional health authorities (Andalucia)
Sweden	Health economics professor and advisor to Tandvårds- och läkemedelsförmånsverket (TLV)
Taiwan	Health economics professor and HTA advisor
Turkey	Health economics professor and advisor to public and private insurance providers
United Kingdom	Health economics professor and advisor to the National Institute for Health and Care Excellence (NICE), Scottish Medicines Consortium (SMC)

RESULTS

- Interviews conducted one-on-one with payer and payer advisors, as well as through an online assessment, demonstrated that payers ranked oncology highest as a therapeutic area in which PRO information impacted decision making, when compared to other disease areas such as central nervous system disorders (neurology or psychiatry), autoimmune disorders, diabetes mellitus or lifestyle (obesity, smoking).
- As part of an assessment to gauge the current and future impact of PRO data on health care decision making, respondents were queried about the impact that PRO data collected after clinical progression has on decision making in oncology.

Perceived Value of Collecting PRO data Post-progression

"Is it worthwhile to collect PRO data in clinical trials for oncology products?"

- All payers/payer advisors interviewed (16 out of 16) replied "yes"
  - accounts for patient perspective (Italy)
  - This is a therapeutic area [Oncology] where QOL information is most important (France)
- All those interviewed indicated that phase 3 and postmarketing trials are the most important phases for collecting PRO data.
- Among on-line survey respondents, oncology ranked highest in terms of impact of PRO information on decision making
- 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

> "Postprogression PRO data could be used if two drugs and one has more/better PRO then more compelled to cover and/or manage less strenuously the drug with PRO data. If marginal endpoint improvement and no PRO data then more likely to manage tightly."
>
> **– US Pharmacy Director** — **US**

- 11 of 16 respondents in one-on-one interviews indicated it is worthwhile to collect PRO data post-progression
  - Most thought it was particularly important for cancer conditions that involved palliative and/or long-term care
- 11 of the 16 respondents in one-on-one interviews indicated that positive post-progression PRO data may support further use of 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

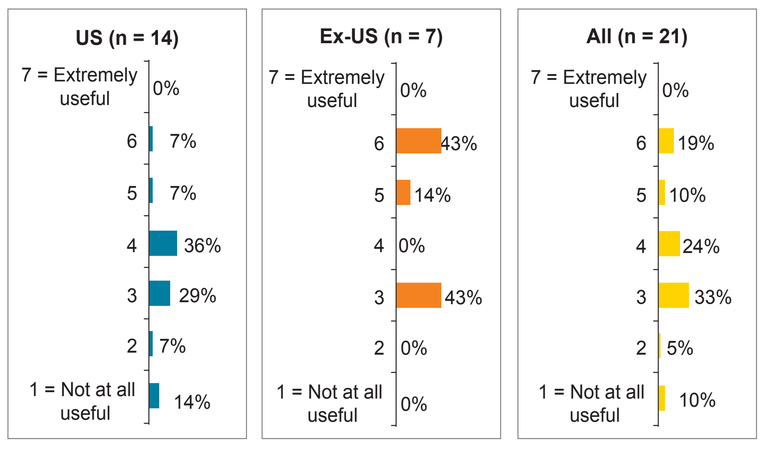
> "…very important, especially in advanced metastatic stage of cancer." — **Poland**

> "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." — **Sweden**

> "….don't like the idea of paying 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 — **US**

- 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**)
  - The usefulness of PROs collected post-progression varied among US respondents

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



Rated on a scale of 1 to 7 where 1 means "not at all useful" and 7 means "extremely useful"

"What characteristics of PRO measures are suitable during the post-progression period?"

- Peer-review publication of data with validated, objective, reliable measures
- Adoption by leading cancer centers, professional societies, or key opinion leaders
- Phase 3 and post-marketing trials with emphasis on comparator trial data and real world clinical experience.

"How long should post-progression PRO data be collected?"

- From one-on-one interviews payers generally deferred to clinical experts on the length and frequency of post-progression PRO data collection.
  - Interview respondents felt this depends on disease progression – as long as possible or at least 1 year
    - Frequency of data collection was also dependent on disease progression
- Online responses were highly variable (9.4 months for ex-US respondents to 13 months for US respondents) with an average of 11.8 months

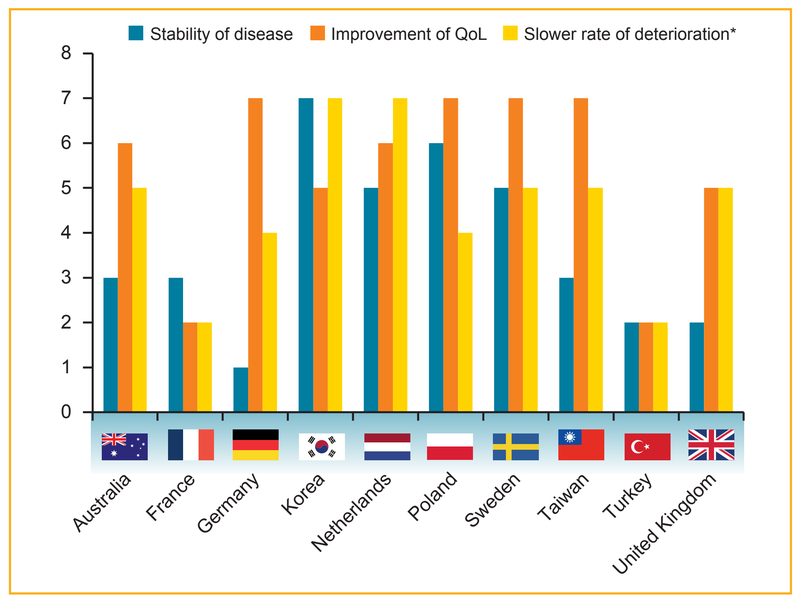
"In what type of cancers would it be useful to collect PRO data while the cancer is progressing?"

- Interview respondents indicated post-progression data is particularly important for cancer conditions that involve palliative and/or longer-term care
- Collection of PRO data was also rated as useful in renal cancer and prostate cancer
- Among the oncology indications provided in the online assessment, a composite ranking of importance of PRO data measured postprogression is
  1. Breast cancer
  2. Tie
     - Non-small cell lung cancer
     - Bladder cancer
  3. Hematological cancers

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

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

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



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

> "…PRO data is valuable in the end-stage of the disease where you are trying to improve QoL…" — **France**

> "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." — **United Kingdom**

> "One of the issues in the assessment of oncology products is the focus on progression free survival and what happens after that is not explored…. it is extremely important to know what is happening post progression…" — **Sweden**

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 progressing**
- **PRO data in oncology will increase in importance over the next 5-10 years, including PRO data measured post-progression.**
- **Post-progression data is particularly important for cancer conditions that involve palliative and/or longer-term care (lung cancer, breast cancer, bladder cancer)**
- **Important post-progression data include stability of disease, health-related quality of life, symptom severity or frequency, functional status and rate of functional deterioration**

REFERENCES

1. DeMuro C. Value Health. 2012;15:443–448
2. Gnanasakthy A. Value Health. 2012;15:437–442

Mobile Friendly e-Prints

Scan this QR code

3 ways to instantly download an electronic copy of this poster to your mobile device or e-mail a copy to your computer or tablet

Text Message (SMS)

Text: **Q4bf57**
to: 8NOVA (86682) *US Only*
+18324604729 *North, Central and South Americas; Caribbean; China*
+447860024038 *UK, Europe & Russia*
+46737494608 *Sweden, Europe*

Visit the web at:
http://novartis.medicalcongressposters.com/Default.aspx?doc=4bf57

Standard data or message rates may apply.

Presented at the ISPOR 18th Annual European Meeting, Milan, Italy, November 7-11, 2015

This study was sponsored by Novartis Pharmaceuticals Corporation.