Patient Experience in Oncology

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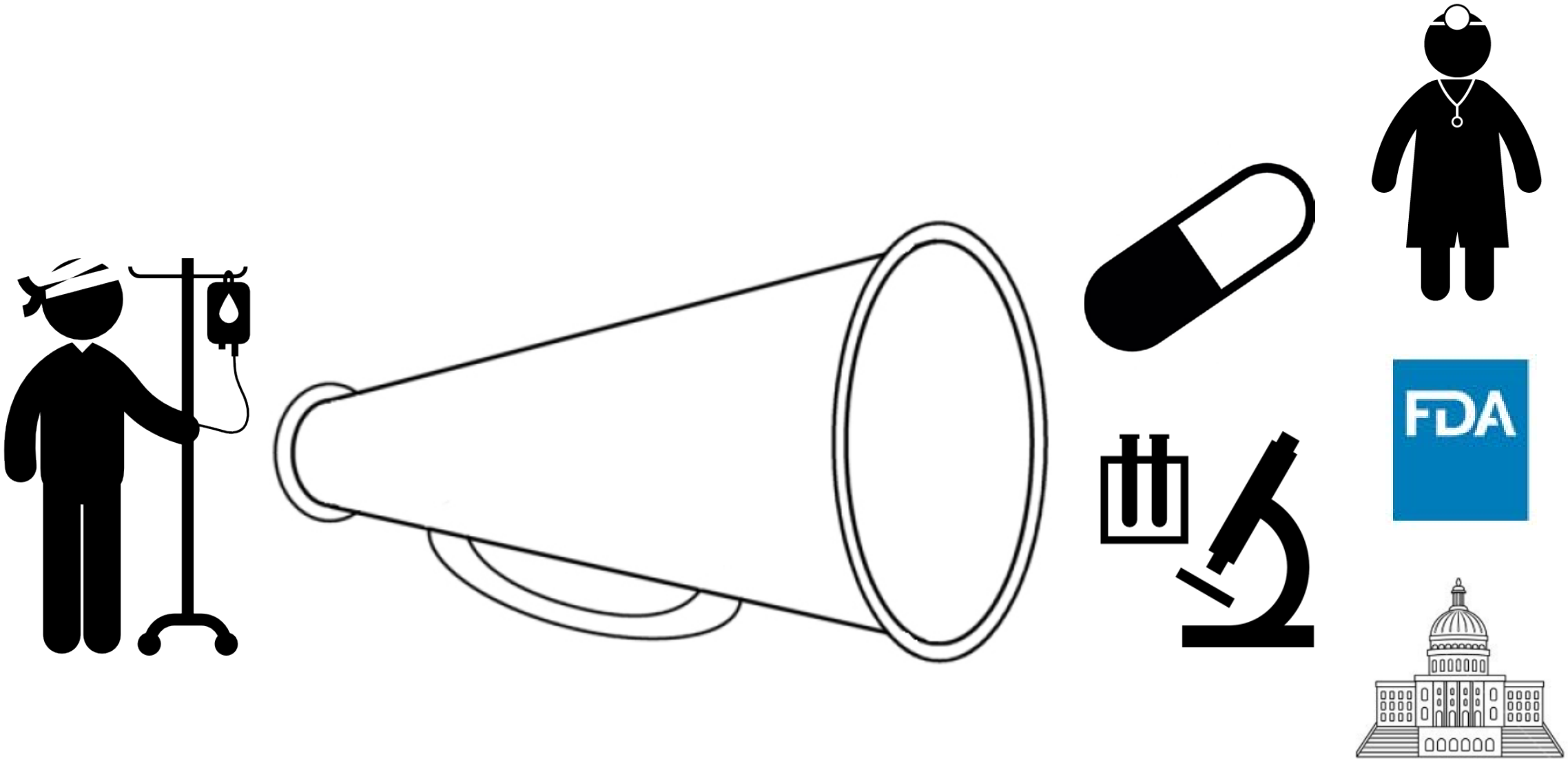
The Patient Voice



What is Patient Experience?

21st Century Cures Act

Patient Experience Data includes data that

(1) are collected by any persons (including patients, family members and caregivers of patients, **patient advocacy organizations, disease research foundations**, researchers, and drug manufacturers); and

(2) are intended to provide information about patients' experiences with a disease or condition, including—

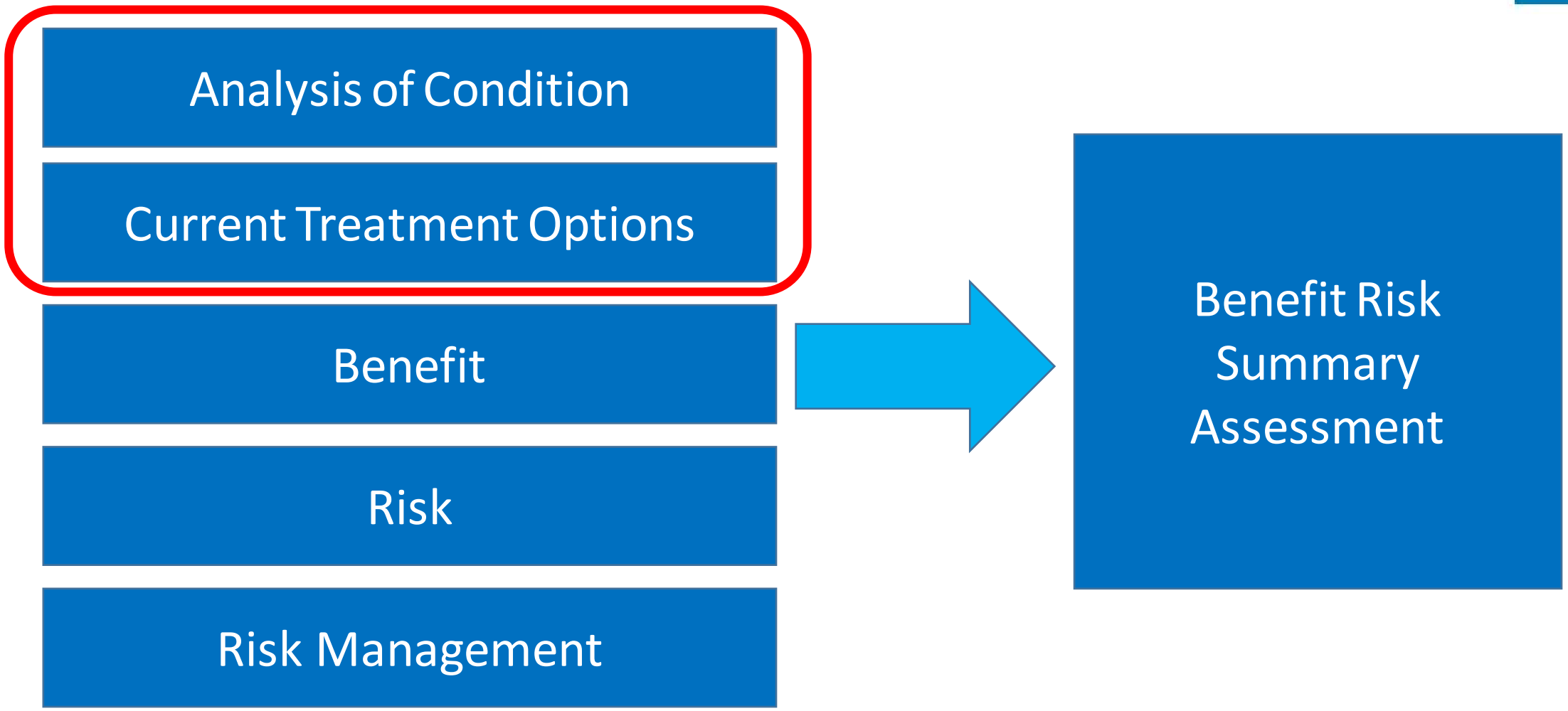
(A) the impact of such disease or condition, or a related therapy, on patients' lives; and

(B) patient preferences with respect to treatment of such disease or condition

Why is Patient Experience Data Important?

- A formal avenue for the patient voice
- Patient experience data can support FDA staff, e.g.,
  - In conducting benefit-risk assessments for products under review, by informing the therapeutic context,
  - Advising drug sponsors on their development program
- It might also support drug development more broadly
  - Help identify areas of unmet need in the patient population
  - Help identify or develop tools that assess benefit of potential therapies
  - Inform endpoint selection and reflect how clinical trials can be designed

Benefit-Risk Framework



Rituxan vs Rituxan Hycela

- **Rituxan™** (rituximab) intravenous initially approved in 1997
  - Currently indicated for oncologic (CLL, NHL) and benign (RA, Granulomatosis Polyangiitis, and MPA) indications
  - Infused slowly over 1.5-4+ hours due to potentially fatal infusion reactions
- **Rituxan Hycela™** (rituximab and hyaluronidase)
  - Submitted for new subcutaneous route of administration for same oncology indications as Rituxan IV
  - Infused subcutaneously into abdominal wall over 5-7 minutes
  - Approved June 2017

PrefMab Trial

- Dedicated, open label, multicenter study to evaluate patient preference SC vs. IV Rituximab
- Patient population: 743 previously untreated patients with DLBCL or FL receiving R-CHOP, R-CVP or R-Bendamustine
- 201 enrolling sites in 32 countries (all ex-US)
- Primary objective: To evaluate the proportion of patients indicating an overall preference using the Patient Preference Questionnaire (PPQ) for either the SC or the IV route of rituximab administration

Patient Preference Questionnaire

Please answer the following questions about your experiences and your preferences. There are not any right or wrong answers.

1) All things considered which method of administration did you prefer?

☐ IV ☐ SC ☐ No preference

2) If you have a preference for one of the administration routes, how strong is this preference?

☐ Very strong ☐ Fairly strong ☐ Not very strong

3) If you have a preference for one of the administration routes, what are the **TWO** main reasons for your preference?

☐ Feels less emotionally distressing

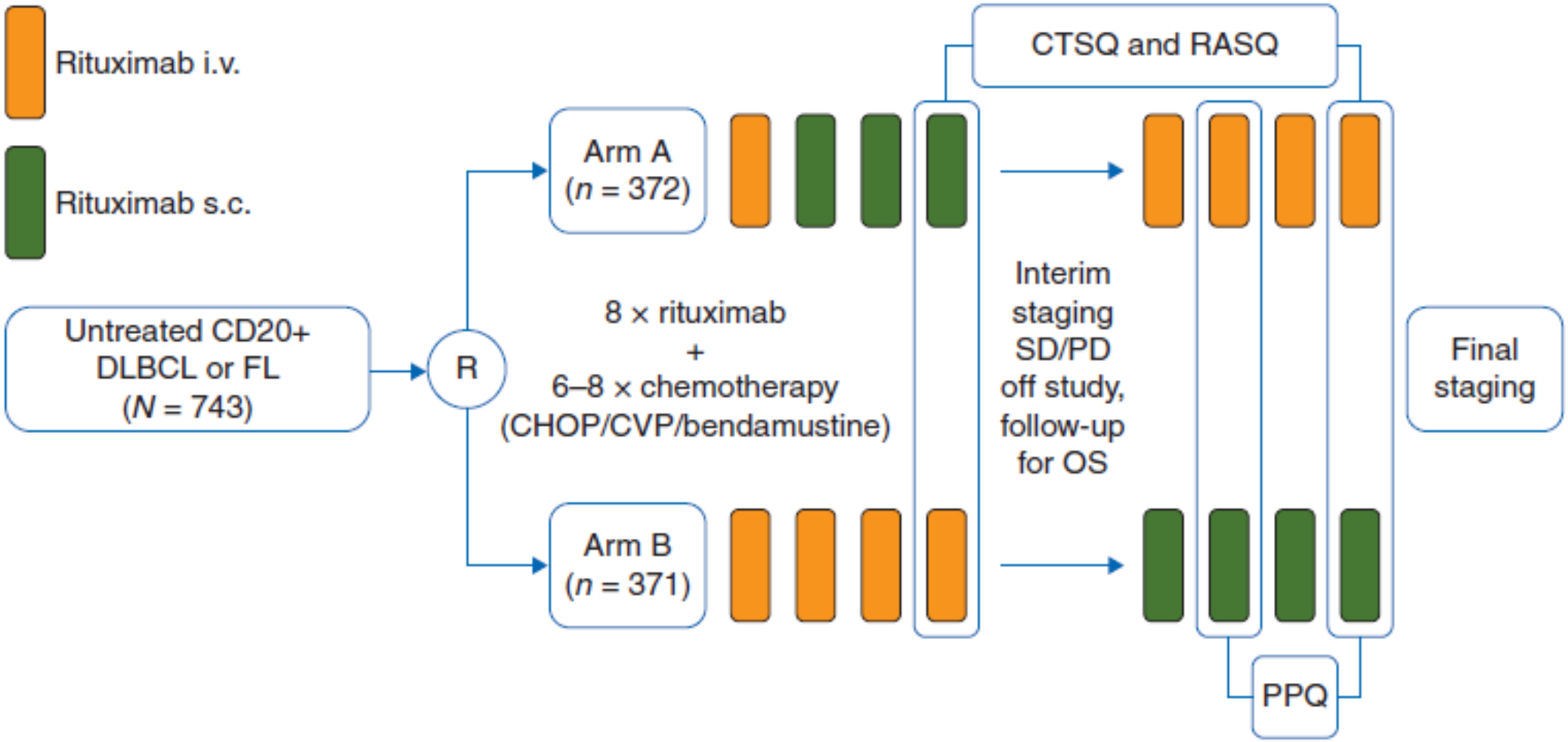
☐ Requires less time in the clinic

☐ Lower level of injection-site pain

☐ Feels more comfortable during administration

☐ Other reason; please specify: .....

PrefMab Design



Source: Rummel, et al. 2017

PrefMab Results

- Patient Preference Questionnaire after Cycle 8:
  - Prefer SC: **77%**
    - Requires less time in the clinic (69%)
  - Prefer IV: **11%**
    - Feels more comfortable during administration (66%)
  - No preference: **7.7%**
  - Did not state a preference: **4.7%**

Rituxan Hycela Labeling

14.4 Patient Experience

Previously untreated adult patients outside of the United States with CD20+ diffuse large B-cell lymphoma (DLBCL) or CD20+ follicular non-Hodgkin's lymphoma (FL) Grades 1, 2, or 3a were randomized to receive a standard chemotherapy regimen (CHOP, CVP, or bendamustine) and either RITUXAN HYCELA 1,400mg/23,400 Units at Cycles 2–4 (after the first cycle with intravenous rituximab) or a rituximab product by intravenous infusion at Cycles 1–4. After the fourth cycle, patients were crossed over to the alternative route of administration for the remaining 4 cycles. After Cycle 8, 477 of 620 patients (77%) reported preferring subcutaneous administration of RITUXAN HYCELA over intravenous rituximab and the most common reason was that administration required less time in the clinic. After Cycle 8, 66 of 620 patients (11%) preferred rituximab intravenous administration and the most common reason was that it felt more comfortable during administration. Forty eight of 620 patients (7.7%) had no preference for the route of administration. Twenty nine subjects of 620 (4.7%) received Cycle 8 but did not complete the preference questionnaire.

Benefits of PE Data

- Patients may not assign the same values to risks and benefits as practitioners and regulators.
- PE data has the potential to capture preference in cases where a discrete choice experiment would be difficult to conduct
- PE data provides practitioners with evidence of patient preference

Regulatory Challenges

- Formalizing the review of PE data
- How to incorporate into “benefits” and “risks”
- Finding the most applicable section of the label to put patient experience data
- How to handle patient experience data that contradicts safety or efficacy data

Good Practices for Collecting Patient Experience Data

- Safety and efficacy between comparators should be well understood
- Instrument should be content valid
- Patient comprehension should be demonstrated
- The study should have adequate sample size and allow for generalizability of results

Take Home Messages

- Patient experience data is useful to patients, caregivers and practitioners
- The FDA will provide guidance for collection and analysis of PE data
- Patient advocacy organizations are well positioned to collect PE data

If your organization is interested in collecting patient experience data: contact the relevant FDA review division