Project PatientVoice

Oncology Center of Excellence

Project Goal

Adhere to the spirit of the 21st Century Cures mandate by creating a web-based public source of patient experience data that is accessible to patients, caregivers, and providers

Background

- 21st Century Cures Act encourages FDA to review and communicate patient experience data submitted in product reviews
- Patient-reported outcome (PRO) data are frequently submitted; heterogeneity exists in analysis and presentation of data
- Product label (USPI) offers limited space to communicate patient experience data adequately

Background

Solution

- Project PatientVoice is a pilot, web-based, public source of PRO data describing patient-reported side effects
- Will develop consistent analytic presentations
- Will partner with sponsors who volunteer to submit their existing trial data for consideration

Drug Trials Snapshots Serves as a Precedent for Project PatientVoice*

Drug	Active Ingredient	Date of FDA Approval	What is it Approved For	Package Insert
ADDYI	flibanserin	August 18, 2015	Treatment of acquired, generalized hypoactive sexual desire disorder (HSDD) in premenopausal women	Addyi
ADLYXIN	lixisenatide	July 27, 2016	Improvement of blood sugar control in adults with diabetes mellitus (DM) type 2 when used in addition to diet and exercise	Adlyxin
AEMCOLO	rifamycin	November 16, 2018	Treatment of traveler's diarrhea in adults	Aemcolo
AIMOVIG	erenumab-aooe	May 17, 2018	Preventive treatment of migraine in adults	Aimovig
AJOVY	fremanezumab-vfrm	September 14, 2018	Preventive treatment of migraine in adults	Ajovy
AKYNZEO	fosnetupitant and palonosetron	April 20, 2018	Prevention of the nausea and vomiting that happens right away or later in adults receiving certain anticancer medicines (chemotherapy)	Akynzeo

Drug Trials Snapshots provide consumers with information about who participated in clinical trials that supported the FDA approval of new drugs. Information provided in these Snapshots highlights any differences in the benefits and side effects among sex, race and age groups. It is part of an overall FDA effort to make demographic data more available and transparent.

Current State

– FDA OCE Patient Focused Drug Development team:
  - Socialized the project across the FDA
  - Presented and received feedback on a mock-up website with visualizations to a group of cancer advocates and patients
  - Finalized agreement with a commercial sponsor to provide data from their clinical trial of approved product
  - Working with sponsor to finalize the presentation of their trial data

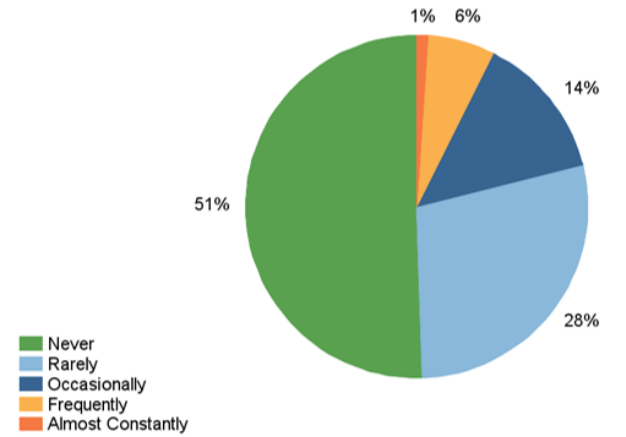
Future State

- Design and deploy the Project PatientVoice home site and visualizations on a public-facing FDA website
- Obtain feedback through a public workshop and other means

Drug	Indication	Trial Name	Study Design	Blinding Status	Number of patients	Number of PRO patients (with baseline)	PRO Tools Used to Measure Side-effect	FDA Label
Cancer Drug	Patients with Advanced/ Metastatic Cancer	Trial A	Randomized	Double Blind	100	100	PRO-CTCAE	Here

Limitations: Project Patient Voice is intended as one of many tools for patients to use when discussing a drug with their physician. Do not rely on PatientVoice alone to make decisions about medical care. Do not use Patient Voice to substitute for advice from your health care professional. Conclusions about patient experiences with side-effects may be limited because the complete drug side-effect profile may not have been captured by the patient-reported survey.

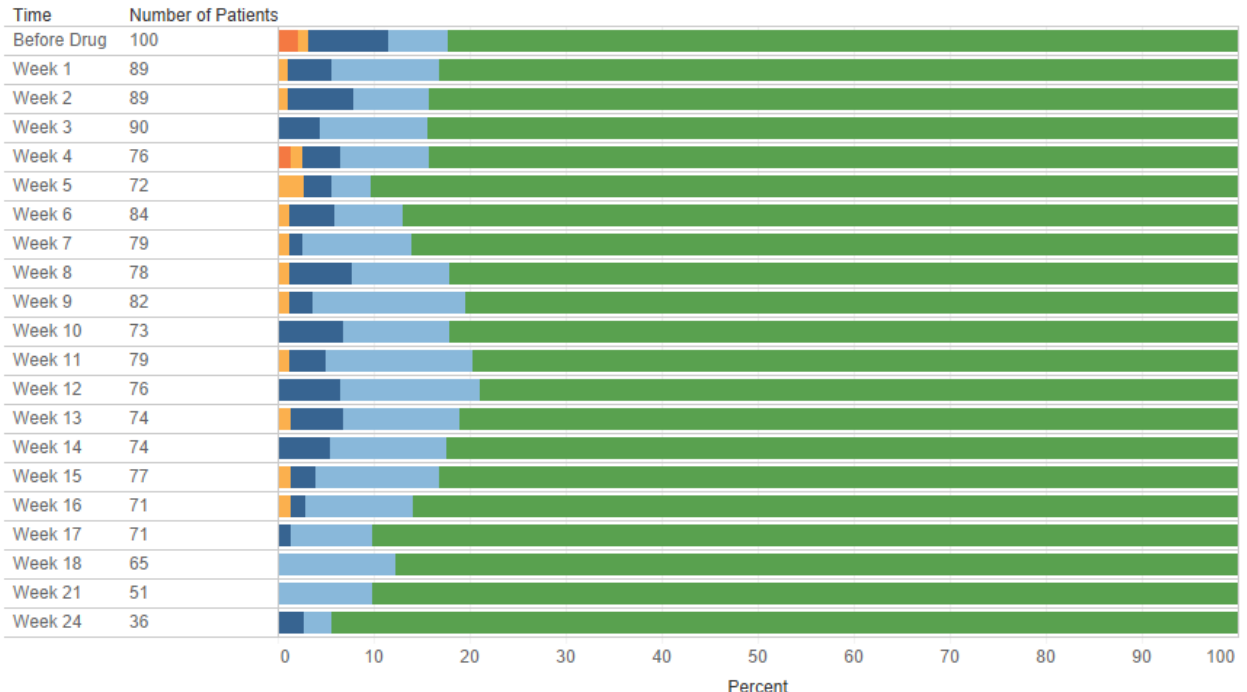
Worst Nausea Score While on Therapy



Worst Nausea Score: This was calculated by finding the worst severity rating score a patient reported any time while the patient was taking the drug

Summary of patient-reported nausea across 6 months of therapy

Question: "In the last 7 days, how often did you have nausea?

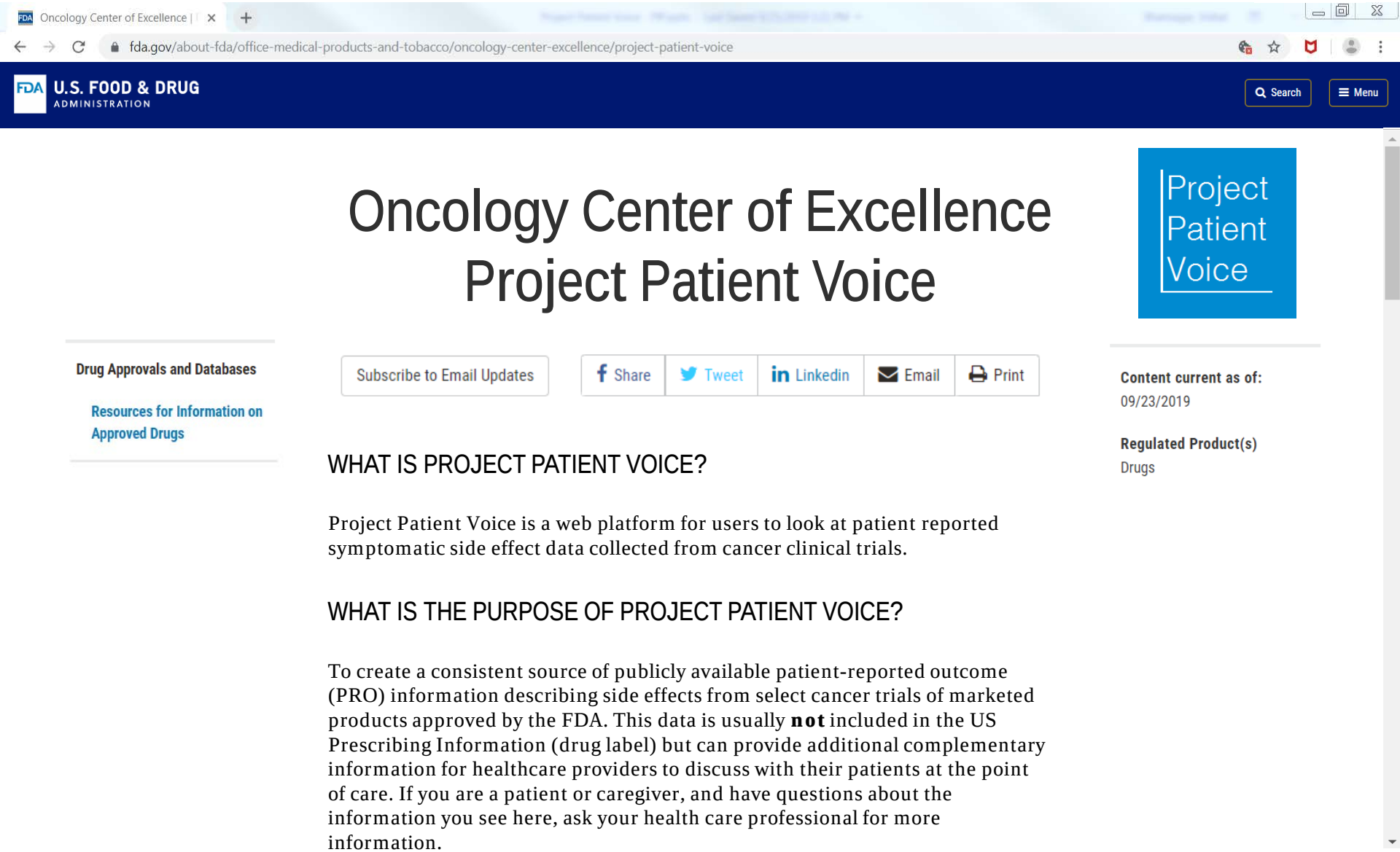


Technical and Data Challenges

- Website design, deployment and maintenance
- 508 compliance (access for people with disabilities) for complex graphs
- Agreement with commercial sponsor on tables and figures
- Providing adequate warning to users on limitations of the analyses (e.g. key side effects may not be captured by PRO surveys and this is not a replacement for clinician reported safety as described in the label)

Lessons Learned (So Far…)

- There is no “perfect” way to display this complex data
- Consistent display over time will allow health care providers to get comfortable with these particular displays
- Different groups (FDA, patient, sponsor, healthcare provider) have different needs, finding creative ways to meet those is required



Subject to change. This is a website mock-up

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Patient-Reported using surveys: Side-Effect Experiences on Cancer Drug

Side Effects	Evaluable Patients	On Treatment Score, Adjusted	
		Any Worsening %	Worsening to 4 or more %
Gastrointestinal Disorders			
Loose or Watery Stools - Frequency	100	50	21
Nausea - Frequency	100	50	21
Vomiting - Frequency	100	50	21
Consitpation - Severity	100	50	21
Mouth & Throat Sores - Severity	100	50	21
Skin Disorders	100	50	21
Acne or Pimples in the Face or Chest - Severity	100	50	21
Dry Skin - Severity	100	50	21
General	100	50	21
Fatique, Tiredness or Lack of Energy - Severity	100	50	21
Arm or Leg Swelling - Frequency	100	50	21

Score	Frequency	Severity
1	Never	None
2	Rarely	Mild
3	Occasionally	Moderate
4	Frequently	Severe
5	Almost Constantly	Very Severe

Patient Survey: Patient Reported Outcome - Common Terminology Criteria for Adverse Events (PRO-CTCAE)

On Treatment Score: To account for side effects reported by patients before receiving the drug, a PRO-CTCAE score during treatment was included in if it was worse than patient's score before they started taking the drug

Note: FDA labeled symptomatic AEs that were not assessed by this survey included nail toxicities, rash, loss of appetite: See FDA label, section 6 for details on the full safety profile of the drug (link)

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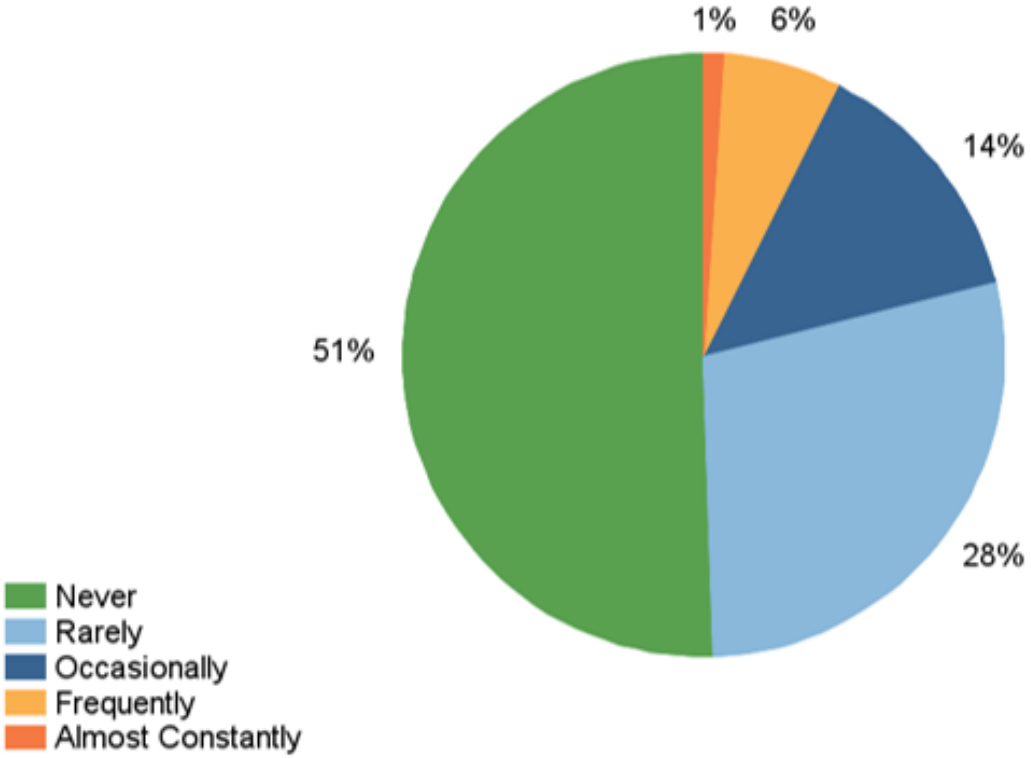

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