SEEKING PATIENT PERSPECTIVES IN CLINICAL TRIAL DESIGN

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THE PATIENT’S VOICE 2017
IMPORTANT CONTEXT

• As a biopharmaceutical business, Amgen is a commercial entity. However, Amgen is also committed to being science based, science driven, and fully compliant with all governing laws.

• This requires the balanced and thoughtful integration of commercial and science-based activities on a strategic level.

• Part of this balance is the proper separation of these activities – from a law/compliance standpoint - in executing science-based activities.

• Amgen’s compliance policies define this appropriate integration and separation.

• The content and spirit of this presentation are intended to be fully consistent with all of the above and with Amgen’s mission to serve patients.

This presentation is intended to capture the vision and aspirations for Patient Centricity with the recognition that laws, rules and regulations of the various countries where Amgen conducts business may differ with respect to development and promotion of biotechnology/pharmaceutical products and interactions with patients, healthcare professionals and entities. Underlying all assumptions in this plan is that all development and promotional activities are guided by a product’s approved indication(s) and product labeling in the applicable country at issue and all relevant country laws, rules and regulations and applicable internal Amgen policies. Amgen will abide by all relevant law, rules and regulations and applicable internal Amgen policies in connection with any development and promotional activities and other interactions with patients, healthcare professionals or entities. Amgen has an effective compliance program in place to ensure such conduct.
OBJECTIVES OF TODAY'S SESSION

• Provide an overview of how Amgen is currently seeking patient expertise to inform trial design and operational planning

• An active session with lots of discussion
  – What is important to consider as we conduct our activities to seek feedback from patients?
  – What will patients expect from us?
  – As we expand the tactics we utilise which ones should we focus on?
AMGEN IS FOCUSED IN 6 AREAS TO INCORPORATE PATIENT VOICE

- Target Product Profile (TPP)
- Dosing Experience
- Clinical Trial Effectiveness (CTE)
- Patient Reported Outcomes
- Regulatory Filing Support (including HTA)
- Product Support Programs
THE PATIENT VOICE PLAYS A KEY ROLE IN ALL STAGES OF DRUG DEVELOPMENT

**Phase 1 & 2**
- Initiate patient voice activities:
  - Understand clinical disease management
  - Identify unmet patient needs
  - Obtain feedback on clinical trial design

**Phase 3**
- Engage patients:
  - Improve recruitment, retention and patient experience on clinical trials
  - Ensure timely clinical trial enrollment
  - Review key data and communication plans
  - Understand gaps in disease state education
  - Understand issues in patient access and clinical use

**Post Approval**
- Continue gathering patient voice:
  - Educate key audiences
  - Identify additional data needs
  - Partner on approaches to ensure appropriate patient access
  - Ensure guideline committees have access to relevant clinical evidence
  - Capture real-world patient data /experience re: drug effectiveness and safety
WHY COLLECT THE PATIENT VOICE IN CLINICAL TRIALS?

• Enhance understanding of patients’ real unmet needs

• Improve trial planning, including developing (more) meaningful endpoints

• Improve the patient experience in clinical trials
  – Lowering the burden of clinical trial participation, improving recruitment and retention
  – Providing channels for two-way communication (patient-sponsor) after a trial
PARTNERING WITH PATIENTS IN TRIAL DESIGN

1. Amgen values patient perspective & engagement
   - Appreciate and honor patients for their clinical trial participation that advances medical science
   - Provide information to patients and their families
   - Actively involve patients in the clinical trial process
   - Partner with clinical trial participants to innovate, and to measure impact and outcomes that are important to patients*
   - Solicit clinical trial participant feedback and respond to it with respect*

2. Amgen values patient experience
   - Seek to understand the clinical trial experience from the patient’s point of view
   - Use appropriate Patient Voice tools for each clinical trial
   - Address patient needs and preferences throughout drug development
   - Help clinical trial sites identify and resolve problems that patients face

3. Amgen values patient health
   - Make medications available to patients post-trial by offering access prior to market availability wherever possible*
   - Communicate trial results to help current patients make appropriate treatment choices and to benefit future patients

**TACTICS TO SEEK FEEDBACK FROM PATIENTS**

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**Facilitated Review**

**Design Approach.** Systematic, patient-centric design approach for clinical research design, operation planning and risk mitigation

- Educate team on Patient Experience
- Facilitated Review Sessions*
- Update Study Design

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**Study Simulation**

**Simulated Visit.** Patients and site staff play ‘roles’ as they simulate (role-play) a study visit or series of study visits (e.g. screening and Visit 1) per the study design and schedule of assessments

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**Patient Panel**

**Moderated Discussion.** A structured, moderated, two-way discussion with patients and/or caregivers

- Direct feedback and insights (written & audio) on clinical study-related areas
- Organized and moderated by an external vendor (3rd party)

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**Online Patient Community**

**Online Forum.** To bring together patients & caregivers to improve clinical development:

- Gain feedback & Collaborate to improve development
- Educate & Build community
- Recruit

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* Focused on Patient journey, leverage points, recommended changes
Business and Patient Value: Enables teams to focus on the patient while designing studies, with foundational knowledge of the patient perspective and behavior and to enhance patient experience.

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<th>Patient Experience</th>
<th>Facilitated Review Sessions</th>
<th>Changes to Study Design</th>
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<td>• Educational Program, synthesis of existing knowledge</td>
<td>• Patient journey, leverage points, recommended changes</td>
<td>• Prioritize changes, plan to incorporate changes by design</td>
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- **Patient Experience**: 
  - Build team knowledge about the patients’ experience with the condition and related behaviors
  - Help the team identify knowledge gaps and prioritize questions we might still need to answer

- **Facilitated Review Sessions**: 
  - Map out the patient journey to provide clarity on the steps patients go through in the trial
  - Enables team to prioritize leverage points & determine drivers around behaviors
  - Convert these insights into recommendations for desired behaviors

- **Changes to Study Design**: 
  - Prioritizing changes based on patient desirability and implementation feasibility will ensure that the changes are incorporated into the study design
Patients and site staff play ‘roles’ as they simulate (role-play) a study visit or series of study visits (e.g. screening and Visit 1) per the study design and schedule of assessments.

The clinical trial is simulated at a real site with real patients, according to the following four key principles:

- **Interventional procedures not conducted, but simulated** (e.g. patient positioned for an x-ray but not actually exposed to radiation).
- **To mimic real life as much as possible, there is no interaction between the Simulation Team and the patient while running the simulation.**
- **Patients complete a questionnaire to establish a basis for comparison.**
- **Semi-structured interviews conducted to identify and focus on the impacts of study participation with the highest degree of salience and relevance for participants.**

**Goal:** To generate deep insights on impact of study design on patients and site staff which can be incorporated into study design and operational planning (see next slide for expansion of benefits).

**Enhance the patient experience**

**Improve the study design**

**Positively influence**

- **Recruitment**
- **Retention**
- **Adherence (Compliance)**
- **Advocacy**
- **Study Effectiveness**
The simulation was a really good experience for us... it was very beneficial to be able to dry-run the protocol.

Study coordinator

During simulation design, site staff expected each visit to take approximately 6 hours. When simulated, the visits actually only took about a half of that time.

The patients were delighted to help and share their perspectives.

Principal Investigator

In the questionnaire, participants told us they felt adequately informed by the informed consent process. However when questioned on elements of the study design they were unable to recall key details.

Nobody has ever asked me that before!

Patient

A wide variety of personality types were encountered during the simulation. Unique and valuable insight was gathered from each of them.

We were able to gather insight from the perspective of both the patient and the site staff on the same patient experience.

As far as I’m concerned, it is not just helpful, but crucial.

Principal Investigator

Patients and parents appeared comfortable disclosing personal circumstances, opinions, and concerns.

STUDY SIMULATION : HOW IT CAN ADD VALUE

There is high perceived value for Patients, Investigators and Research Staff
**IMPROVE STUDY DESIGN & MATERIALS**

**Motivation, Enrollment & Retention**
Incorporating patient feedback can improve study design to reduce patient burden, optimize patient-facing study materials for both the patient and the sponsor, improve patient understanding and participation and therefore increase enrollment and retention rates.

**UNDERSTAND THE PATIENT EXPERIENCE**

**Walk in the Patients’ Shoes**
Patient Panels give patients an opportunity to provide feedback directly to sponsors on a variety of clinical study-related areas. The valuable insights gained from patient panels are then used to improve future study volunteer experiences.

**KNOW YOUR PATIENTS**

**Validate Patient Insights**
The goal of a Patient Panel is to learn more about patients’ motivations, past experiences and current opinions to better understand their perception of products and clinical trials and to learn how specific product or clinical trial characteristics might impact patient experiences and choices.

**PATIENT PANEL**

**Business and Patient Value:** Patient Panels provide valuable insights to the product/program/study team by bringing together patients, caregivers and/or members of the healthcare community to engage in conversation and share experiences.
ONLINE PATIENT COMMUNITY

Business and Patient Value: Offers a single forum to engage patients and/or caregivers in many ways to gain feedback on improving development

Online Forum to engage & connect. Bring together patients and/or caregivers to engage in conversation, share experiences & link to resources.

Connection & Community
Engage with patients sharing similar experience.

Resources
Access advice & connect to resources.

Clinical Trial Community
Access information & data. Participate in research.

An Online Patient Community can be combined with other patient engagement methodologies, for example social listening, digital journaling, digital focus groups & co-creation.
THE ONLINE PATIENT COMMUNITY OFFERS UNIQUE BENEFITS FOR ENGAGING PATIENTS IN CLINICAL DEVELOPMENT...

Forum to bring together patients and/or caregivers to gain feedback on improving development:

- **Patient Benefit** – On-demand access to information and tools, ability to share ideas / feedback in real time & engage with other patients, flexible access via multiple modalities possible.

- **Expanded Reach** – Communication with a diverse, geographically-dispersed population.

- **True Patient Voice** – Direct, unfiltered, real-time voice where “anonymous” status (if applicable) promotes candid responses & ideas [limits can be applied]

- **Rich Info Resulting in Detailed Insights** – Adequate time available to probe responses. View into patient world & environment to understand patient perspective, experience & drivers. Ability to create and administer multiple subcommunities.

- **Longitudinal** – Conversations can occur over weeks or months, enhancing ability to gain true patient voice.

- **Effective & Efficient** – Rapid recruitment & implementation possible. Comprehensive channel for distributing & collecting information (eg, release study updates and patient experience questionnaires).
Online Patient Communities can be very effective, when the team is focused on…

- **Entry into Therapeutic Area.** Build depth on patient journey and gain insights into patient experience, needs, patient relevant outcomes and tradeoffs in therapy decision.

- **Clinical Trial Design.** Gain insights on patient experience and needs relevant to interest and participation in clinical trial. Identify key factors & potential candidates for site selection. Solicit feedback on and co-create trial design.

- **Clinical Trial.** Manage trial recruitment, consent & administration. Provide trial information. Track visits, therapy adherence, health data and patient experience. Feedback on lay summaries and future improvements to trial design.

- **Patients that Thrive Online.** Thirst for connection. Active online. Participate anytime, anywhere.

- **Expanded Reach & Scope.** Geographically dispersed. Patients able to track data (adherence, symptoms, biometrics) and record via community portal.

...AND IS RECOMMENDED AS VERY EFFECTIVE FOR SPECIFIC OPPORTUNITIES

Digital format of Online Patient Community offers wide geographic reach, rapid access to patients, flexible options for engaging with patients and capturing data, resulting in rich insights.
FOR DISCUSSION

• What is important to consider as we conduct our activities to seek feedback from patients. What hasn’t worked well in the past and why?

• What will patients expect from us?

• As we expand the tactics we utilise which ones should we focus on and how should we measure for success?