Developing Recommended Language on Patient Engagement for Consideration by FDA

February 22, 2017

Agenda

- Welcome & Introductions
- Review of Progress (Activities to Date)
- Release of Recommended Language for FDA Consideration
- Plans for 2017
- Q&A

Activities to Date (1/2)

March 2015: NHC & GA co-host forum for stakeholders at FDA

September 2015: NHC & GA release Dialogue/White Paper from March meeting

December 2015: NHC & GA convene stakeholder meeting to inform scope and contents of recommended language for FDA consideration

April 2016: Dec. 2015 Meeting Summary released

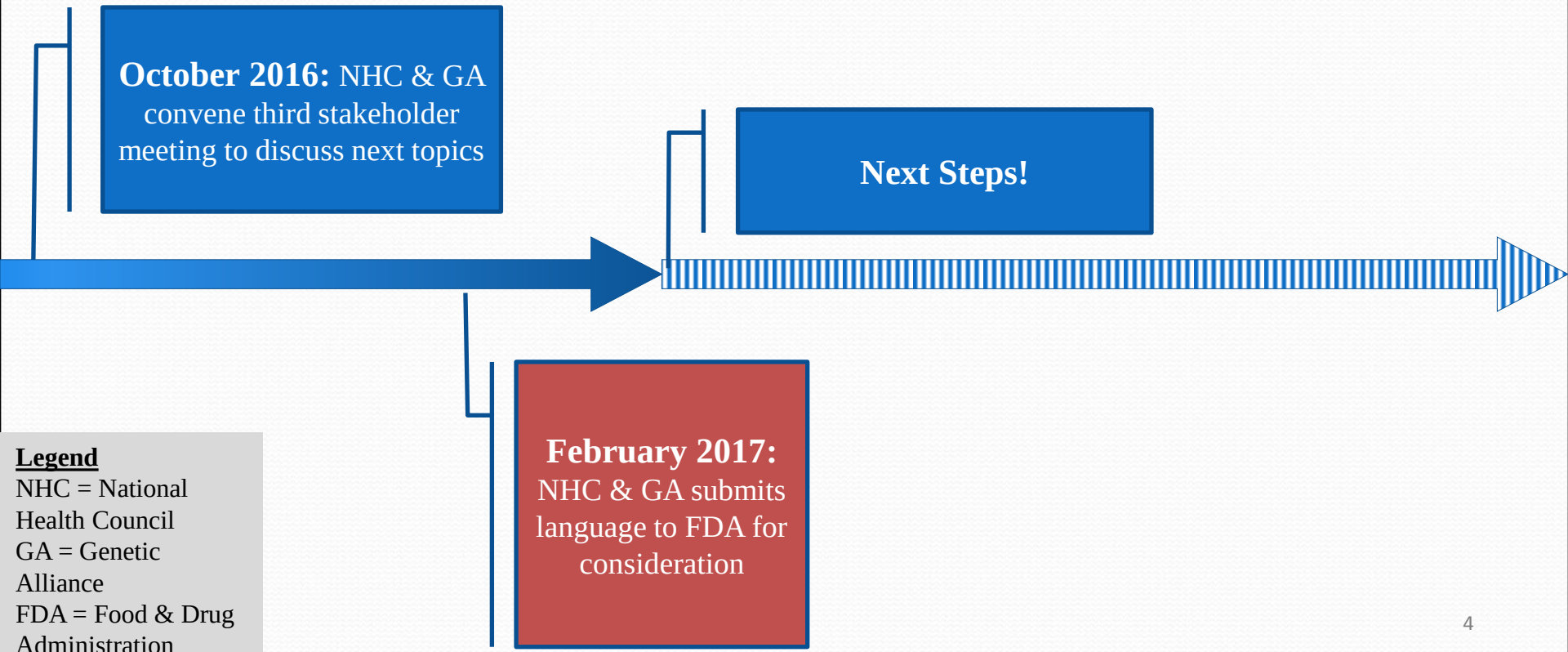
June 2016: NHC & GA release a draft of recommended language for FDA consideration

Summer 2016: Stakeholder comments received on draft of recommended language

Legend

- NHC = National Health Council
- GA = Genetic Alliance
- FDA = Food & Drug Administration

Activities to Date (2/2)



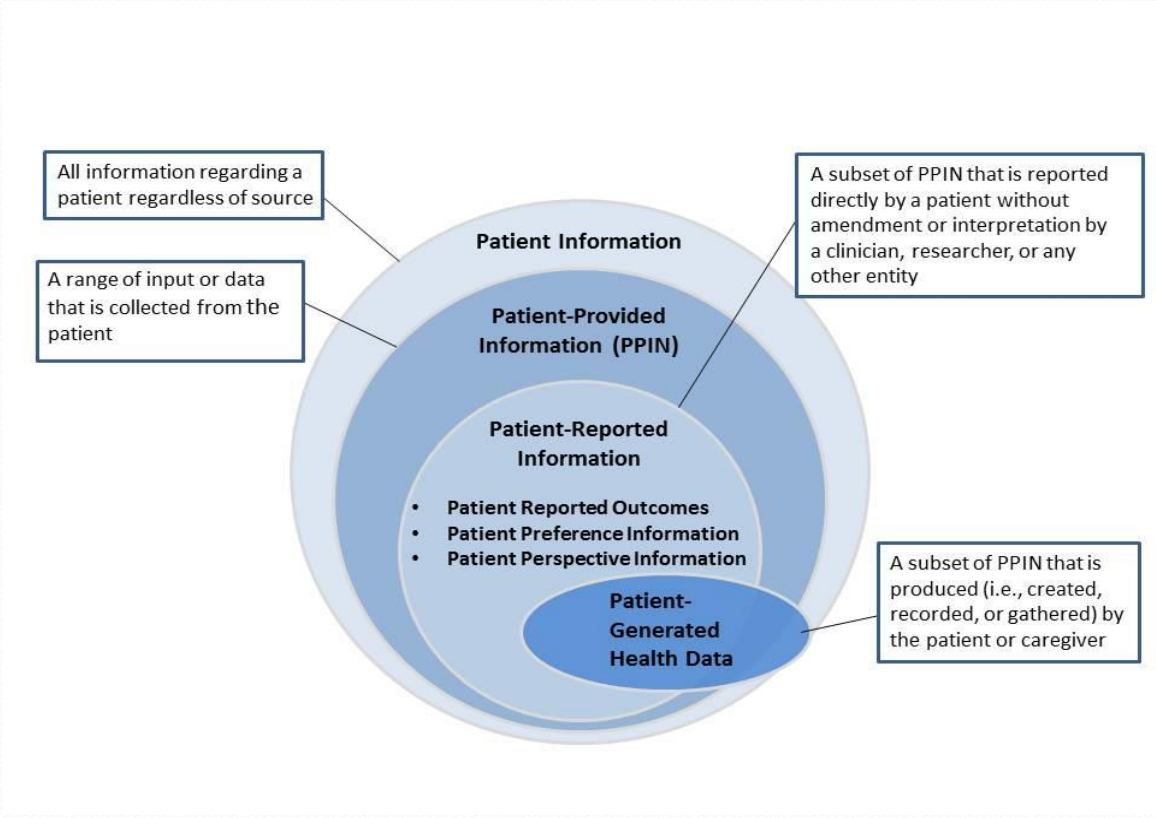
Legend
NHC = National Health Council
GA = Genetic Alliance
FDA = Food & Drug Administration

Recommended Language for FDA consideration

Encouraging and Defining Patient Engagement in Drug Development

- Contains recommended language for FDA consideration in future guidance documents.
  - Provides definitions for various terms related to patient-focused drug development to encourage standardization.
- Conveys to the public that the FDA supports and encourages, but does not require, patient-focused drug development activities.

Relationship Among Types of Patient Information



Plans for 2017

Future recommended language topics for FDA consideration will focus on:

1. **Approaches to collecting patient input on burden of disease and current treatments**
2. **Approaches to collecting input on outcomes most important to patients**
3. Approaches to identifying and developing measures for an identified set of impacts that can be used in clinical trials
4. Who to engage to collect patient-provided information

Plans for 2017 (cont.)

5. Determining when to engage patients throughout the product development process to collect patient-provided information
6. Opportunities to engage with FDA on patient-provided information during drug development and product application submission
7. Representativeness

Next Steps

- Early March: Form Two Working Groups
- March – May:
  - Bi-monthly calls to develop recommended language for each topic
  - Develop drafts
- June/July: Stakeholder review
- August: Final versions for public release
- Add two more working groups by the end of summer

How to volunteer

Send an email to Fred McElwee at fmcelwee@nhcouncil.org with the following information:

- Name
- Title
- Organization
- Type of Organization
  - Patient or patient group, pharmaceutical company, trade organization, professional organization, not-for-profit, academia, government, other)
- Email
- Telephone
- Mailing address
- Work Group Preferences:
  - 1.
  - 2.

Related Activities

- Conference on emerging good practices in patient engagement
- Roundtable on defining representativeness

Q&A

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