



# HCV Drug Access for People with Bleeding Disorders Roundtable - 10/17/2011

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Mark Antell and Paul Brayshaw

Principals, People with Bleeding Disorders & HCV

[Accesshcvtherapy@gmail.com](mailto:Accesshcvtherapy@gmail.com)

<http://sites.google.com/site/accesshcvtherapy/start>



# The Crisis

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- **HCV is “the leading cause of death” in our community.**
- HCV is ubiquitous among people over 30 years old with bleeding disorders who use replacement clotting Factor.
- Advanced disease is common (“advanced fibrosis ~25%”).
- Poor response to SOC therapy may be due to duration of infection.



# The Rationale for Treatment Protocols to Include People with Bleeding Disorders

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- Patients and clinicians have established a long relationship and understand our crisis.
- National Network of Hemophilia Treatment Centers located within teaching hospitals provides specialized care in collaboration with infectious disease clinics.
- Population adheres to therapy regimes.

# Recent History



- Citizen Petition to FDA in 2009
- FDA response included:
  - Hearing to discuss unmet medical needs
  - Guidance for early testing of DAA combinations
  - Support for 'treatment protocols' including bleeding disorders
  - Reduced biopsy requirements for people with bleeding disorders to participate in clinical trials
- Industry
  - Early testing of DAA combinations have demonstrated low side effects and high efficacy.



# Advocacy Objectives

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- Early access to safe and effective HCV treatment for our community and those with special unmet needs.
- Special attention to those who have a poor prognosis and poor medical alternatives.
- Recognition that the need is urgent and requires urgent measures.

# Advocacy Objectives for Today



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- Discuss how to include people with bleeding disorders and others with special unmet needs following success of early phase 2 drug combinations.
- Begin defining a process to develop and submit 'curative treatment protocols' that include people with bleeding disorders and special unmet needs, knowing that success
  - will save lives immediately,
  - will enhance the credibility of applications for drug approval and general access.

# Advocacy Objectives for Today



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- Describe safety/efficacy criteria to trigger an expanded access protocol request for FDA approval.
- Describe resources and infrastructure required to manage curative treatment protocols for special unmet needs
- Discuss alternative pathways to early curative treatment



# Recognition of the Therapy Development Team

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- Forum for Collaborative HIV Research
- Bleeding disorders community-based organizations and clinicians
- FDA in their actions to address unmet medical needs
- Pharmaceutical Industry for their aggressive pursuit of drug development and greatly improved therapy options