

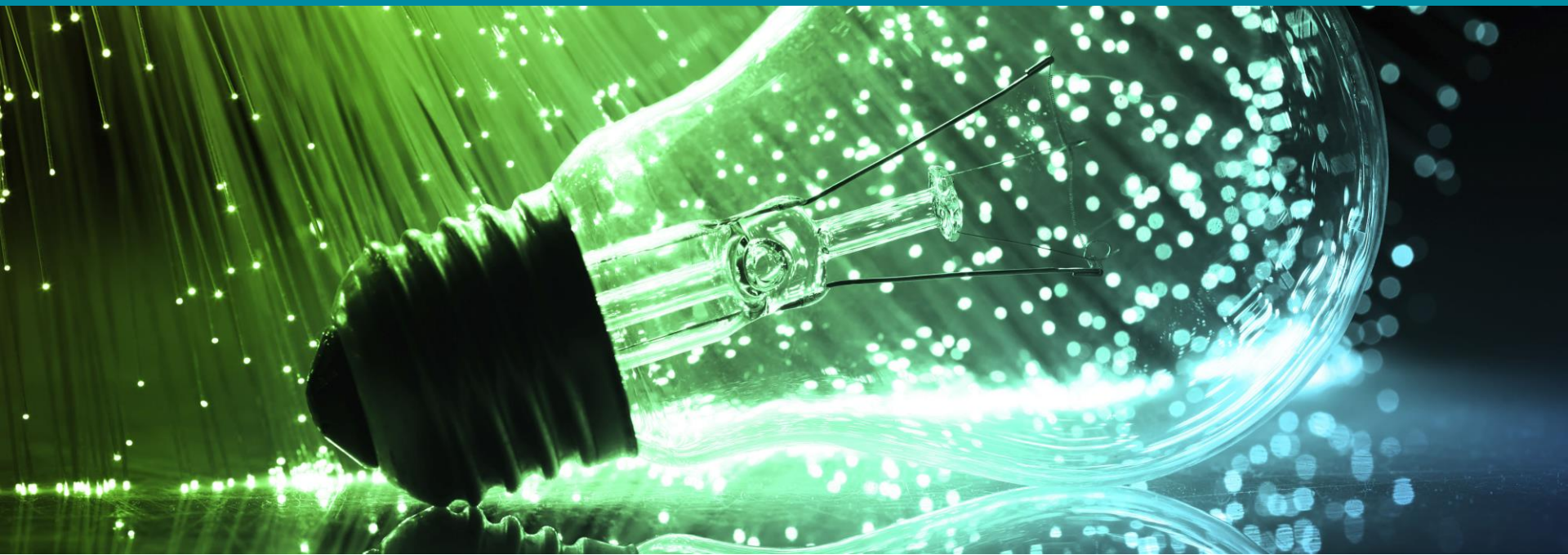
# Session III: Key Issues & Considerations

Facilitator: Rose Tiernan, FDA

*March 1, 2016*



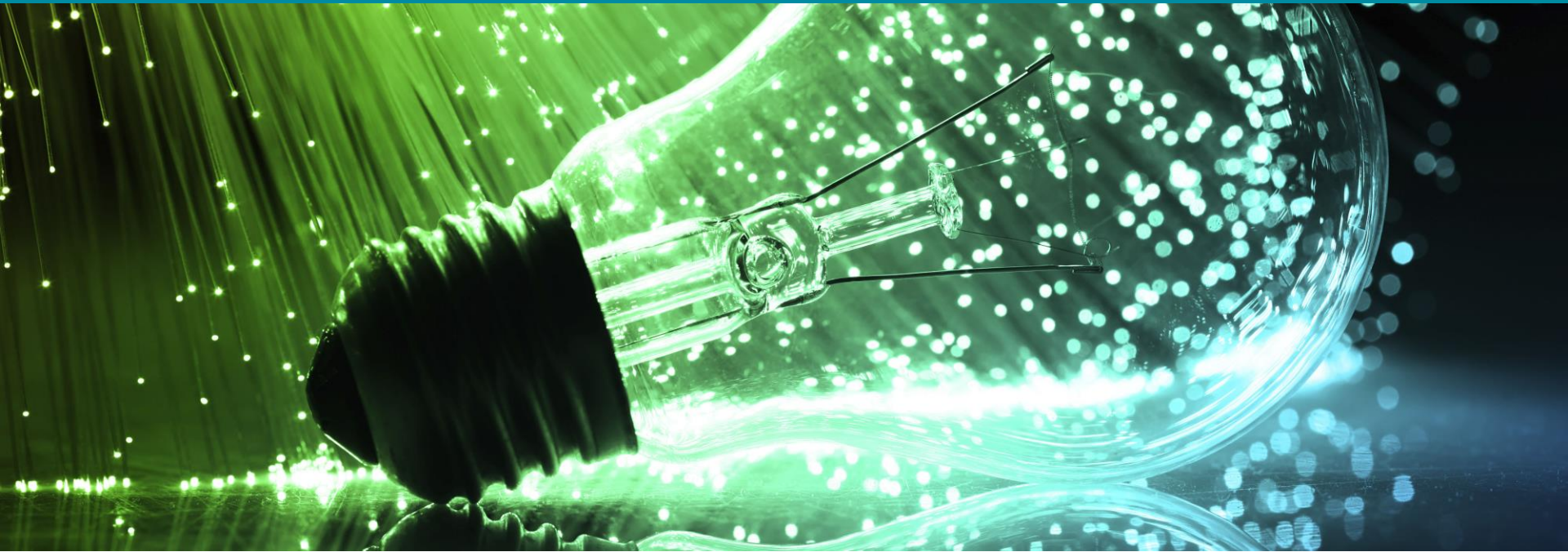
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## **Disclaimer: Rosemary Tiernan, MD, MPH**

The views and opinions expressed in this presentation are those of the speaker and do not reflect official FDA, HHS or other government opinion or policy. They also do not necessarily reflect the views of the Clinical Trials Transformation Initiative.

I have nothing to disclose.



## **Presentation of Focus Group Issues**

Stephen Mikita, JD, Patient Advocate, Team Lead

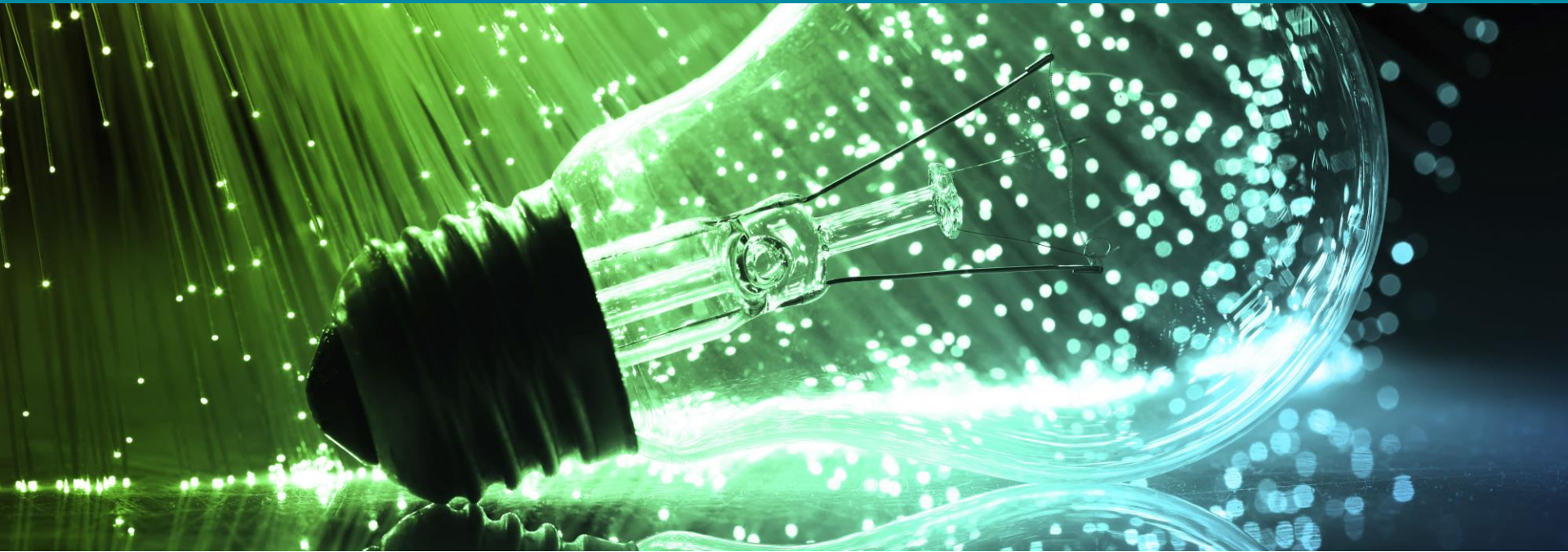
# Session Objectives

## Discuss Key Issues from Patient & Provider Focus Groups:

- Gaps & Challenges?
- Implications for Stakeholders?
- Solutions?

## Issues

1. Patients/Providers perceive antibacterial drug development crisis
2. Clarifying definition of “unmet need”
3. Limiting overuse/inappropriate use of streamlined development approaches
4. Decision making and medical-legal risk issues
5. Stewardship
6. “Real-time” post-approval data collection, data sharing, and publication of treatment guidelines for use of these products
7. Risk Communication



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# Key Issue #1: Patients/Providers Perceive Crisis in ABDD

- **Patients/Providers**—Acknowledge the need for *streamlined development approaches*.
- **Patients/Providers**—Both Providers and Government (regulators) *share responsibility* in developing programs/approaches to address unmet need.
- **Patients/Providers**—Recognize the need for **effective**, not just rapid, steps to be taken to **reduce the risk** of MDR bacterial infections, especially in *hospital and long-term care facilities*.

## Key Issue #2: Patient Understanding of Unmet Need in Antibacterial Drug Development

- ▶ **Patients** need *further clarity* on the *definition* of **unmet need**.
- ▶ **Patient** advocacy groups (e.g., oncology) have various understandings of unmet need – **limited vs. no options**.

## Key Issue #3: Limit Overuse/Inappropriate Use of Streamlined Development Approaches

- **PATIENTS** believe these approaches are necessary but *desire safeguards to prevent misuse by industry.*
- **PROVIDERS** believe these approaches are warranted and **also believe:**
  - The process should not be abused & need reassurance that incentives are commensurate with severity of need.
  - Regulators should maintain adequate oversight of these streamlined development approaches to ensure scientific rigor.
  - Stewardship should be encouraged.

## Key Issue #4: Decision-Making and Medical-Legal Risk

- ▶ **PATIENTS** desire input and consensus from a multi-disciplinary team
  - *The “A-Team”*
  - Ensures that providers are making the right decisions regarding approved or off-label use.
  
- ▶ **PROVIDERS** are concerned they may be at *increased medical/legal risk* when using drugs developed through these approaches
  - Need assurance that using these approved drugs, where the safety database supporting approval may be more limited, does not place them at additional medical-legal risk.

## Key Issue #5: Stewardship

- ▶ Limit use of antibacterial drugs developed with streamlined approaches
- ▶ **PROVIDERS:** Unless there are extenuating circumstances, reserve use of antibacterial drugs developed using these approaches *only* following known culture results.
- ▶ **PATIENTS:** There may be a need to increase public awareness of how decisions are made regarding unmet need, treatment timeliness, and diagnostic limitations.

# Key issue #6: Require “Real-Time” Data Collection/Sharing and Publication of Treatment Guidelines

## PATIENTS and PROVIDERS

- desire ongoing information to be collected and made publicly available regarding antibacterial drugs developed using these approaches.
- besides post-marketing studies, one approach to providing this information might be to implement a registry for drugs developed using these approaches.

## Issue #7: Risk Communication

- ▶ Provide standardized information to prescribing physicians in hospitals
- ▶ The following information should be readily available to physicians with hospital prescribing privileges:
  - Is the drug on the hospital formulary?
  - Are there any restrictions to prescribing the drug (who, when)?
  - What is the cost of the drug?
  - What is the known data in the critically ill?
  - What are the known renal effects? Other known side effects?

# Thank you.



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