

Ahead of the Curve –
Emerging CF Therapies 2009:
North American Cystic Fibrosis
Conference
October 16, 2009

Peter J. Mogayzel, Jr., MD, PhD

Associate Professor of Pediatrics

Director, Cystic Fibrosis Center

Medical Director,

Pediatric Lung Transplantation Program

Johns Hopkins Cystic Fibrosis Center

The Johns Hopkins University School of Medicine

Disclosure

Peter J. Mogayzel, Jr., MD, PhD discloses that he has no financial relationship with commercial supporters.

Other Role: CF Director - Novartis Pharmaceuticals, Genentech, Inc.

Conference Goals

- Engage you in an interactive learning experience to help better identify the potential therapeutic targets of current clinical research.
- Summarize the known data regarding ongoing research.
- Integrate information about these emerging therapies into current clinical practice to more effectively manage patient expectations.

Learning Objectives

After attending this activity, the you should be able to:

- Describe the most current understanding of the pathophysiology underlying CF.
- Identify the potential therapeutic targets of current clinical research.
- Summarize the known data regarding on-going research into CFTR modulators
- Recognize the current research regarding salt transport/correcting ion transport, P2Y2 agonists, ENaC antagonists, et al.
- Integrate information about these emerging therapies into current clinical practice strategies for managing patient expectations.

Ahead of the Curve – Emerging CF Therapies 2009: Pathophysiology and Therapeutic Targets

Richard B. Moss, MD
Department of Pediatrics
Stanford University

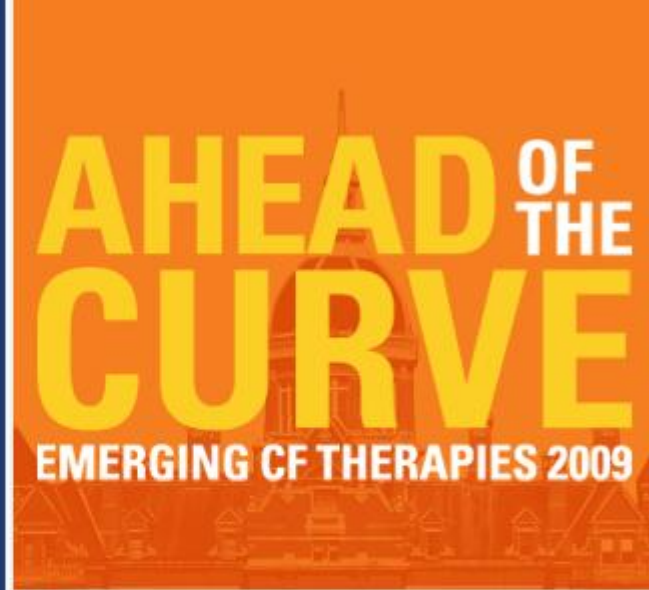


Ahead of the Curve – Emerging CF Therapies 2009: Ongoing Research into CFTR Modulation

J.P. Clancy, MD

Department of Pediatrics, and
The Gregory Fleming James Cystic Fibrosis
Research Center

The Children's Hospital of Alabama and
The University of Alabama at Birmingham



AHEAD OF THE CURVE

EMERGING CF THERAPIES 2009

Ahead of the Curve – Emerging CF Therapies 2009: Who needs CFTR?: Alternatives to Activation of the CFTR for Therapeutic Intervention in CF

Pamela L. Zeitlin, MD, PhD

Professor of Pediatrics

Co-Director, the Johns Hopkins Cystic Fibrosis
Clinical Center

Deputy Director, Institute for Clinical and
Translational Research

Director, Eudowood Division of Pediatric
Respiratory Sciences



Ahead of the Curve –
Emerging CF Therapies 2009:
Managing Patient Expectations
Patients' Case Scenarios

Christopher H. Goss, MD, MSc
Associate Professor of Medicine
University of Washington



Disclaimer

In order to present this program, we applied for and received an unrestricted educational grant from Vertex Pharmaceuticals. What that means is we independently developed the course and then sought funding from Vertex. They had no input or influence, whatsoever, on today's presentations, or who was chosen as faculty.

In addition, the program is vigorously vetted by The Johns Hopkins CME Department and the content is peer-reviewed to ensure that the program is free of commercial bias.

Pre-Seminar Knowledge Assessment

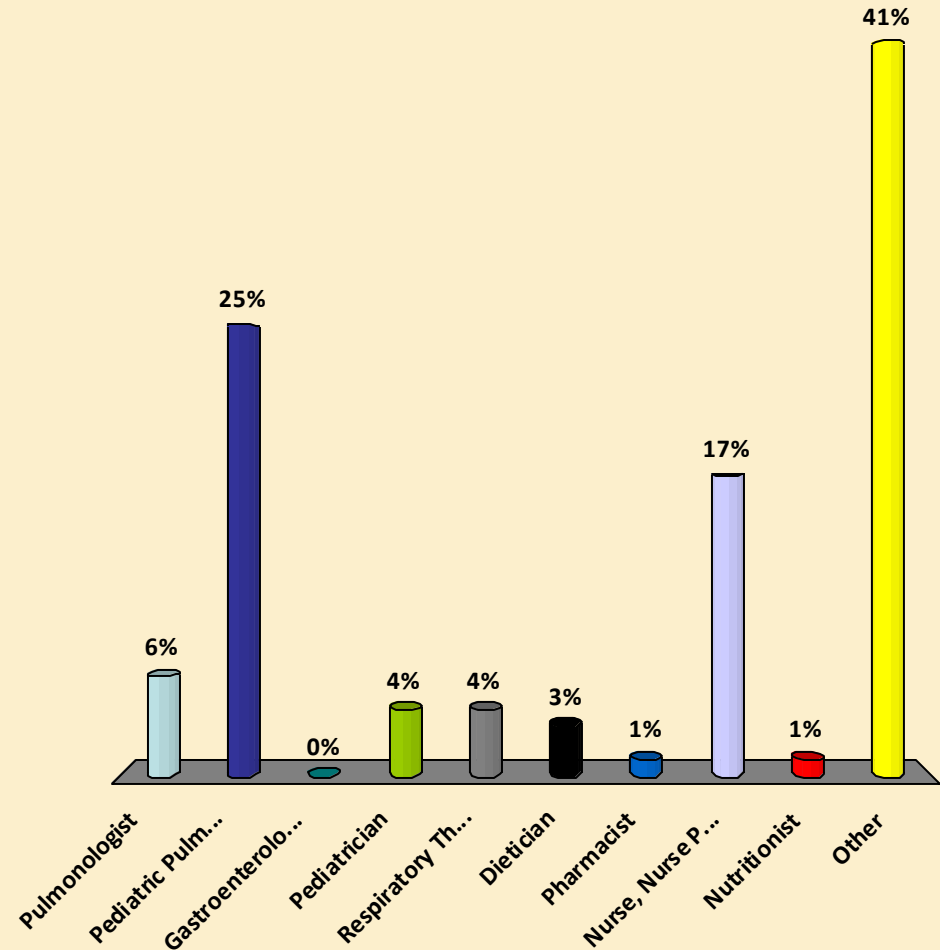
Earlier this year we surveyed many of you to prepare for this seminar and help identify perceived knowledge gaps.

Based on those responses we tailored the upcoming presentations to meet your needs and set the conference goals.

Now, with the ARS, audience response system, we want to do a quick assessment.

Please indicate your occupation

1. Pulmonologist
2. Pediatric Pulmonologist
3. Gastroenterologist
4. Pediatrician
5. Respiratory Therapist
6. Dietician
7. Pharmacist
8. Nurse, Nurse Practitioner
9. Nutritionist
10. Other



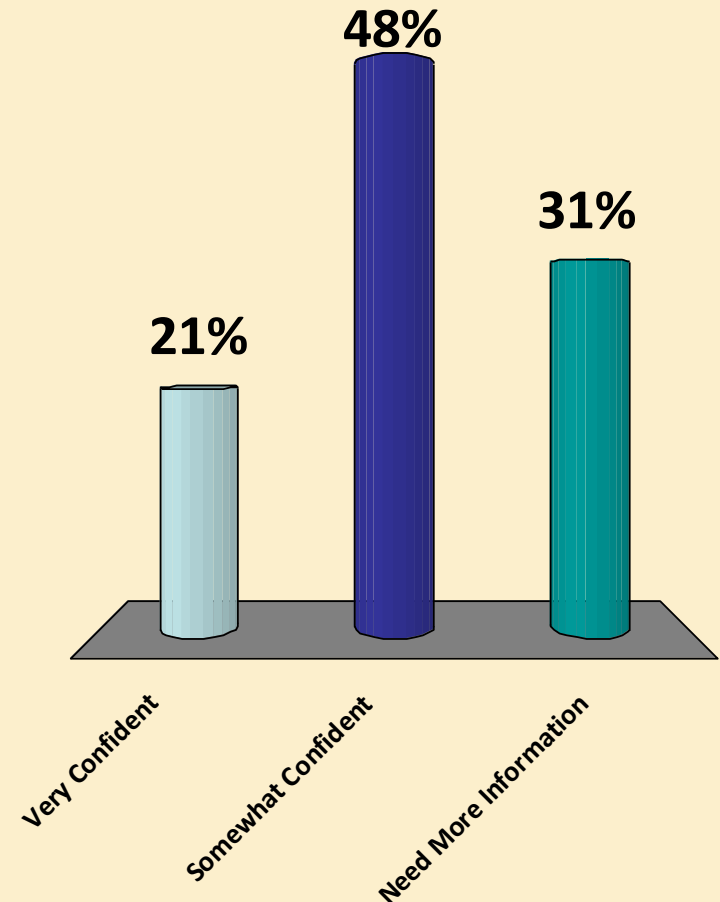
ARS KNOWLEDGE QUESTIONS

Pre-Seminar Knowledge Assessment

Please indicate your confidence level regarding knowledge of ongoing research in:

CFTR Modulation

- A. Very Confident
- B. Somewhat Confident
- C. Need More Information

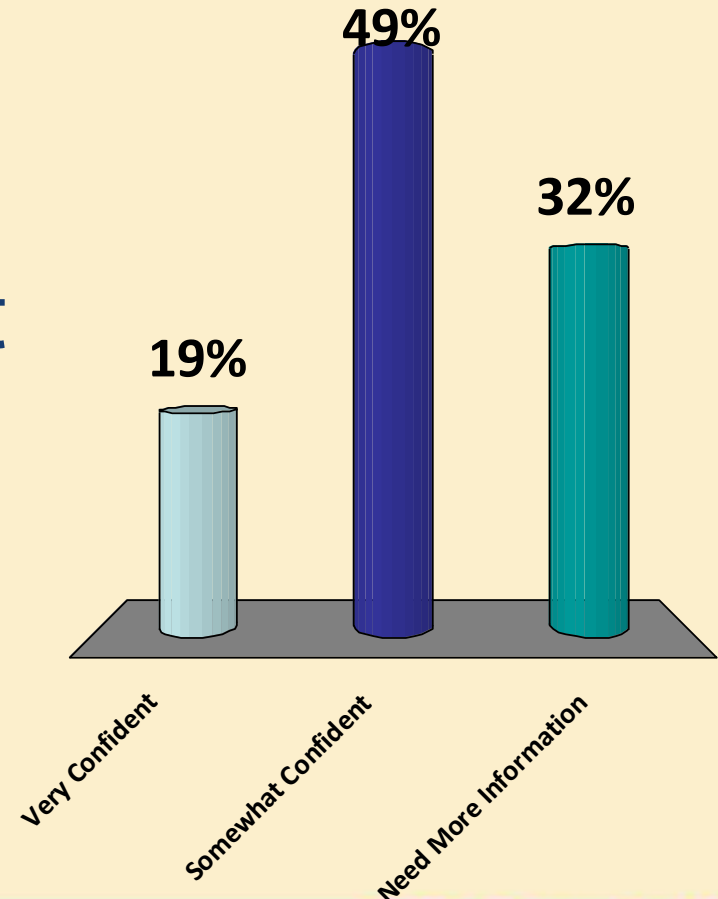


Pre-Seminar Knowledge Assessment

Please indicate your confidence level of knowledge of ongoing research in:

Salt Transport and Correcting Ion Transport

- A. Very Confident
- B. Somewhat Confident
- C. Need More Information

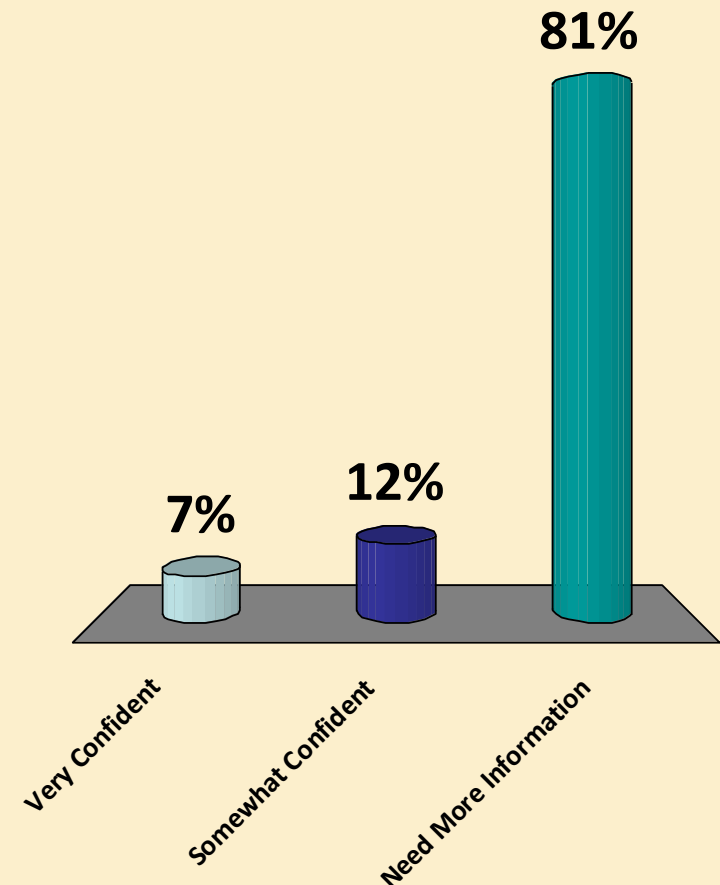


Pre-Seminar Knowledge Assessment

Please indicate your confidence level regarding knowledge of ongoing research in:

P2Y2 Agonist

- A. Very Confident
- B. Somewhat Confident
- C. Need More Information

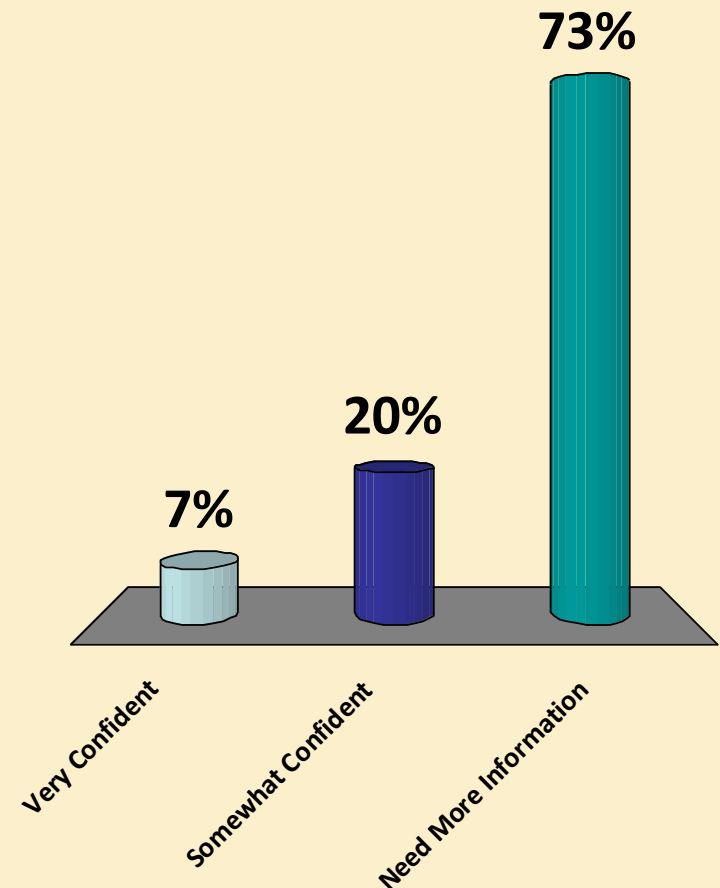


Pre-Seminar Knowledge Assessment

Please indicate your confidence level regarding knowledge of ongoing research in:

ENaC Antagonists

- A. Very Confident
- B. Somewhat Confident
- C. Need More Information

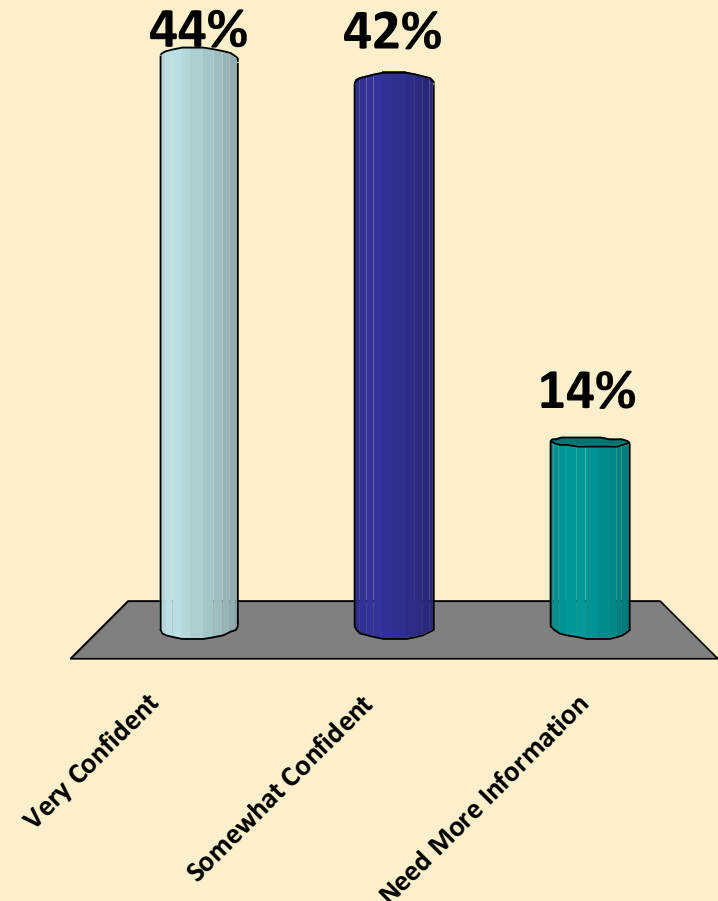


Pre-Seminar Knowledge Assessment

Please indicate your confidence level regarding knowledge of ongoing research in:

Mucus Clearance

- A. Very Confident
- B. Somewhat Confident
- C. Need More Information



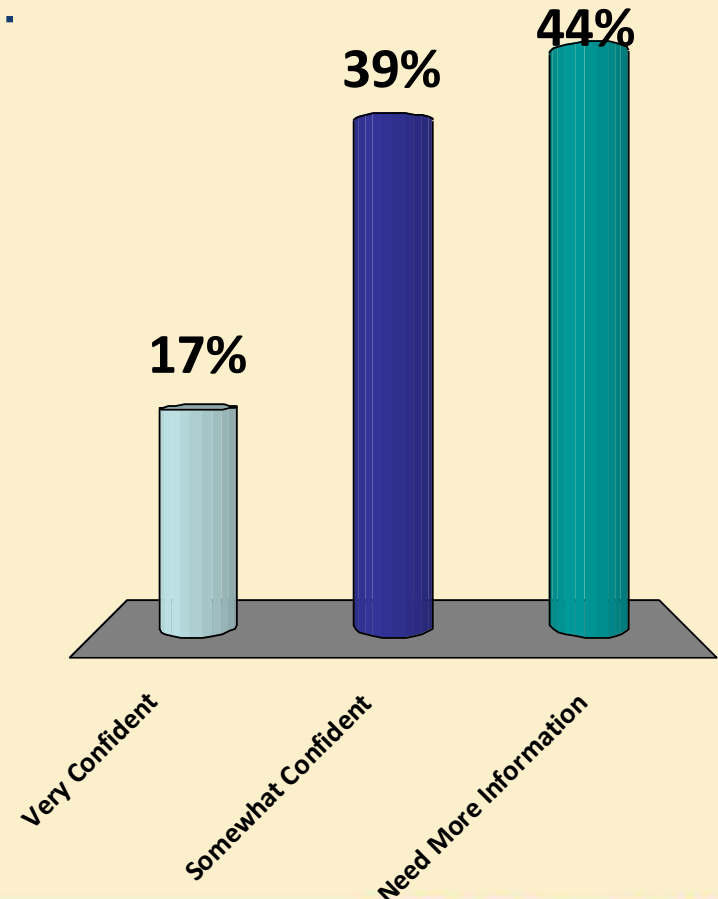
ARS CONFIDENCE LEVEL QUESTIONS

Pre-Seminar Confidence Assessment

Please indicate your confidence level **in discussing with your patients** knowledge of ongoing research in:

CFTR Modulation

- A. Very Confident
- B. Somewhat Confident
- C. Need More Information

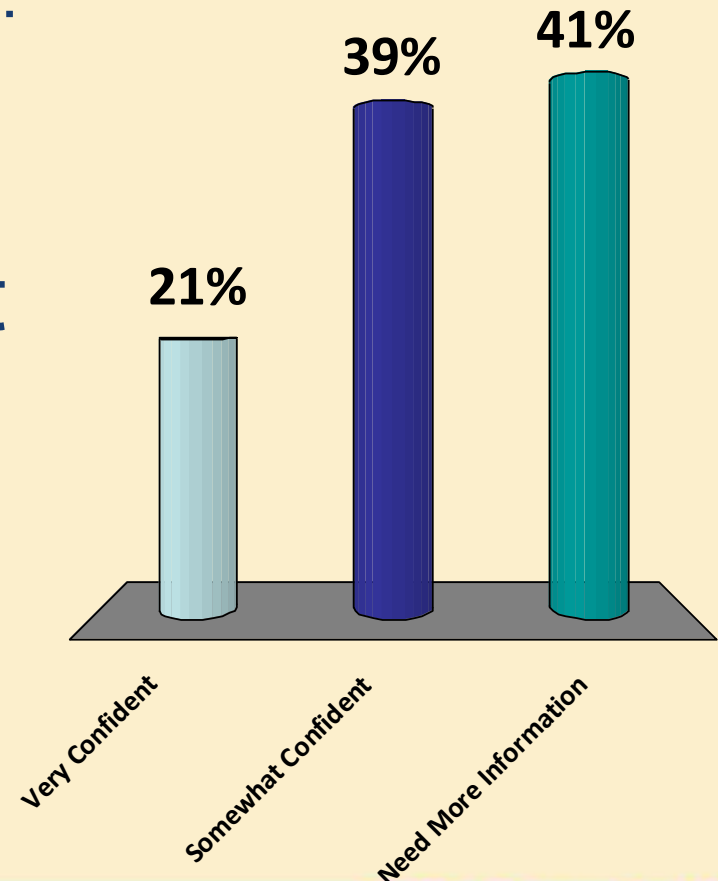


Pre-Seminar Confidence Assessment

Please indicate your confidence level **in discussing with your patients** knowledge of ongoing research in:

Salt Transport and Correcting Ion Transport

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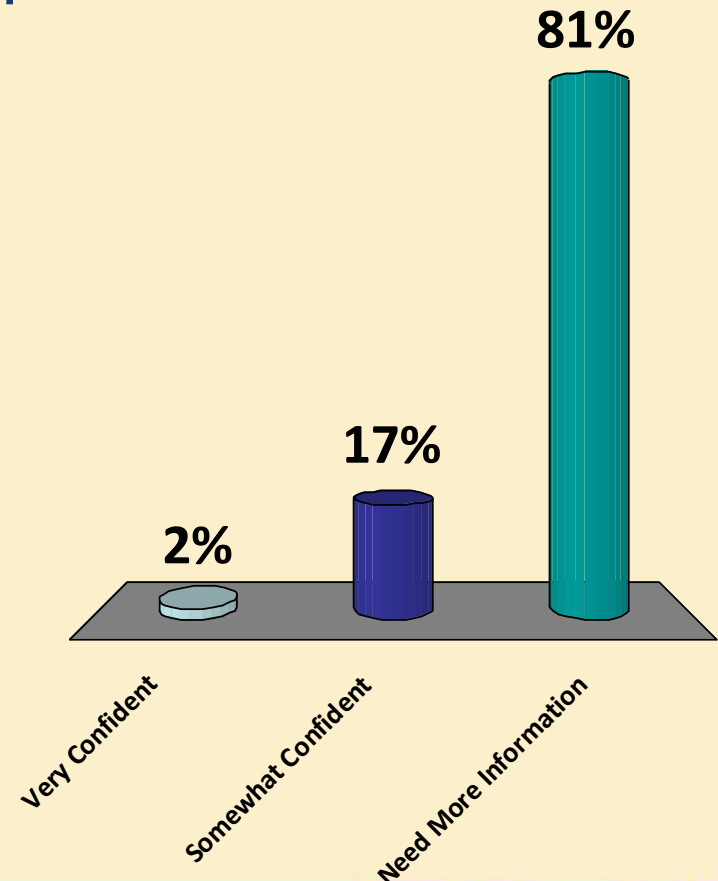


Pre-Seminar Confidence Assessment

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P2Y2 Agonist

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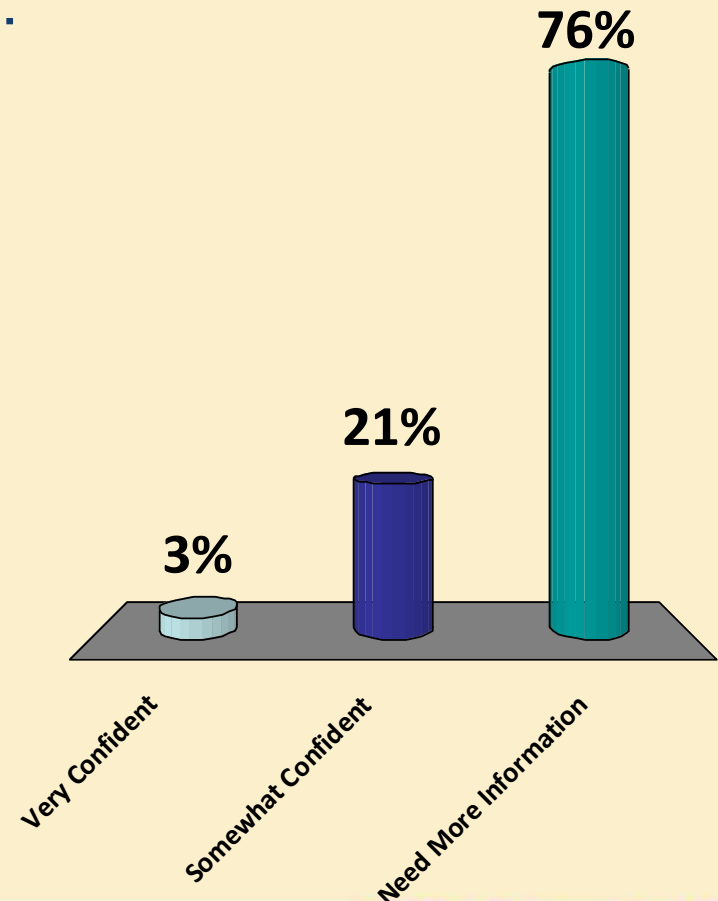


Pre-Seminar Confidence Assessment

Please indicate your confidence level **in discussing with your patients** knowledge of ongoing research in:

ENaC Antagonists

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Pre-Seminar Confidence Assessment

Please indicate your confidence level **in discussing with your patients** knowledge of ongoing research in:

Mucus Clearance

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