NCCN Oncology Summit: Recommendation for REMS Stakeholders

May 7, 2010
National Press Club
Washington, DC

www.nccn.org

National Comprehensive Cancer Network®

Presenters

Peyton Howell, MHA
President, Consulting Services & Health Policy
AmerisourceBergen Specialty Group

Scott Gottlieb, MD
Resident Fellow
American Enterprise Institute for Public Policy Research

Discussion Points

- **Standardization of REMS**
- **Development of REMS programs**
- **Assessment of the impact of REMS on:**
  - Patient outcomes
  - Prescribing patterns
  - Access
- **Medication Guides**
- **Off-label drug use**

"Ultimately we are looking at patient safety, but how can that be accomplished if there is not a central tracking requirement on these patients?"
- Pharmacist

NCCN Trends™ Survey, March 2010

Standardization

- **Complex REMS (i.e., those with ETASU) are not standardized or centralized and have program-specific:**
  - Certification (and re-certification)
  - Training and enrollment
- **Creates inefficiencies in the administrative process of registering and enrolling in REMS programs that require it**

Standardization

- **The current process for developing and approving a REMS program is inefficient**
- **FDA does not routinely solicit and incorporate provider feedback into the final REMS**
- **Could result in:**
  - Delay of access to medications
  - Suboptimal uptake and delayed adoption of an innovative therapy after reaching the market
  - Inability of providers to integrate REMS procedures if they are contrary to existing oncology practice.

Standardization of REMS processes to allow for the provision of efficient care

Near term	Medium term	Long term
Manufacturers should work together to develop common procedures for certification, training, and enrollment in REMS programs (i.e., share "best practices")	Convene a summit with all stakeholders to develop common definitions and procedures for centralization and standardization of REMS registries	Work with health information technology companies to develop a central clearing-house or web portal for REMS and integrate REMS into EHR

Manufacturers face many challenges when developing REMS programs

1. Communication with FDA and other stakeholders regarding the development of such programs
2. Methods to assess a successful implementation
3. Inability to hold providers accountable for following REMS requirements

Recommendations to improve the development of individual REMS programs

Near term	Medium term	Long term
REMS applicants should include providers as part of the discussion while in the development phase	The FDA should standardize core survey questions	Develop a public-private advisory committee that includes clinical professional and patient advocacy societies to guide the development of REMS programs
Manufacturers and the FDA must communicate early in the clinical development process regarding risk strategy	A manufacturer's work group should be convened to provide input and help finalize future REMS guidance	Utilizing scientific methods, refine the methodology behind implementing risk mitigation strategies

Medication Guides

- Technical language
- Risk vs. benefit discussion
- Relevance to individual treatment situation
- Assessment of patient understanding

Recommendations to improve the Medication Guide

Near term	Medium term	Long term
Medication Guides should be reformatted to address benefit as well as risk.	Medication Guides should be pre-tested prior to use to gauge comprehension and literacy.	If so desired, allow for providers to customize (to a certain extent) the Medication Guide to reflect specific risks pertinent to the population being treated

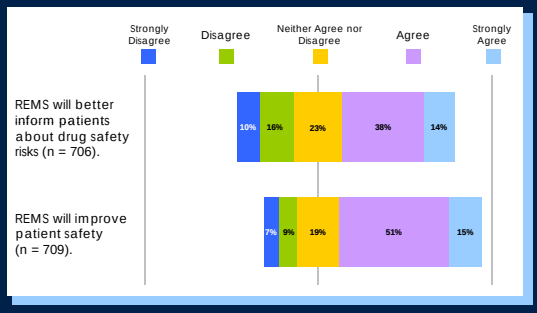
Assessment of REMS

- Clinical decisions are based on evidence using scientific methods
  - Practice of evidence-based medicine
  - Institutional quality improvement initiatives
- Scientific basis for the assessment of REMS
- Look to FDA for guidance on proper methods

Assurance that REMS programs improve patient safety

Near term	Medium term	Long term
FDA must define "success" so that manufacturers can develop appropriate REMS programs and measurement tools.	Conduct a comprehensive survey of providers and qualitatively assess the perceived impact of REMS on patient safety	Perform a long-term study that assesses whether adherence to REMS requirements improves patient outcomes (safety)

Providers are optimistic that REMS will improve patient safety



NCCN Trends™ Survey, March 2010

Off-Label Drug Use and REMS

- **REMS: mitigate specific toxicity (sometimes in specific indication)**
  - Follows product labeling
- **Significant acceptable off-label drug use in oncology**
  - Intended use does not match REMS
  - Use of ESAs in patients with myelodysplastic syndrome (MDS)

Impact of REMS on off-label prescribing

Near term	Medium term	Long term
	FDA must provide more guidance for manufacturers on off-label drug use issues arising from REMS.	FDA should consider allowing manufacturers under a REMS to provide complete information on off-label drug use in order to inform physician/patient decision-making.

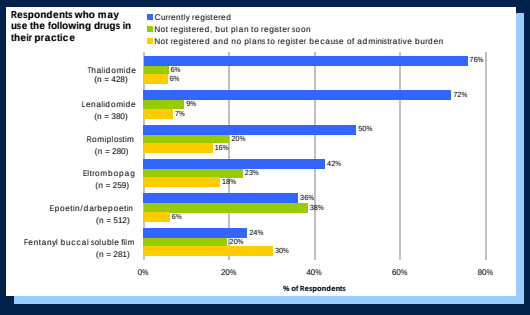
Summary

- Manufacturers, providers, the FDA, and other stakeholders should collaborate to standardize REMS processes
- Development and assessment of REMS programs should be more "scientific"
- Patient safety – metrics for evaluation
- Impact of REMS on off-label prescribing

Considering Unintended Consequences

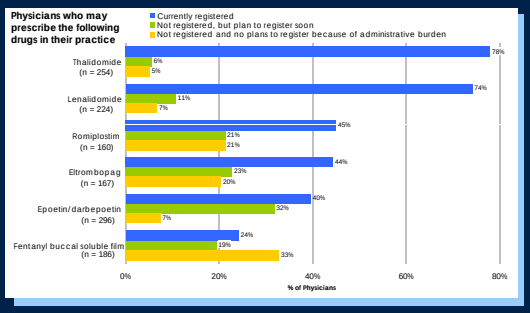
- Shift of utilization to drugs without REMS
- Access
- Disparities in care

Overall participation in REMS programs for specific oncology drugs with ETASU

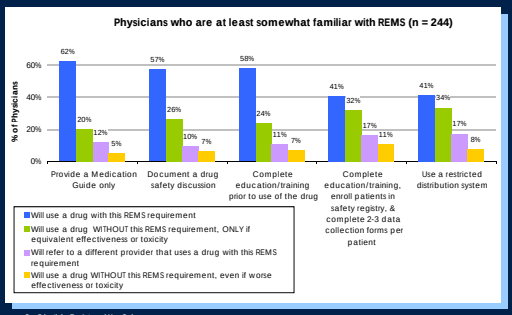


NCCN Trends™ Survey, March 2010

Physician participation in REMS programs for specific oncology drugs with ETASU

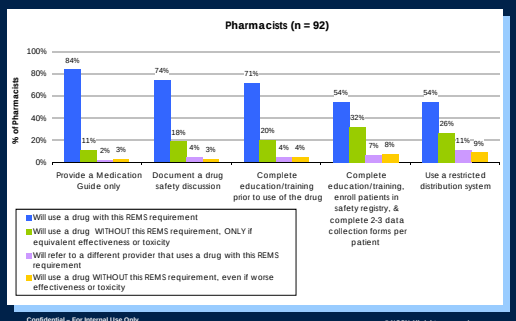


REMS requirements and perceived impact on prescribing



NCCN Trends™ Survey, March 2010

REMS requirements and perceived impact on dispensing



NCCN Trends™ Survey, March 2010

"I am registered for 4 drugs as they are essential to our patient population, but I am aware of patients in other practices who are not getting these valuable treatments [because] their providers do not choose to register…Just today I had another oncologist's office staff call and ask if I would prescribe for their patient, who I had never seen, because they did not want to get registered but they did not want to refer the patient and lose them to their practice. I politely declined because I could not evaluate the patient, assess appropriateness for therapy and monitor response."

- Nurse Practitioner

NCCN Trends™ Survey, March 2010

Roundtable Panel

- Rekha Garg, MD
- Scott Gottlieb, MD
- James Hoffman, PharmD
- Peyton Howell, MHA
- Emily Mackler, PharmD
- Brenda Sarokhan, MPH

Stay Tuned…Part 2

- Provider viewpoints and recommendations
- Patient considerations