Prototype Development and Study Design

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Overview

- Fictitious drug
- Prototype development
- Study design

Fictitious Drug Example: Rheutopia (arixalate)

- Previously used for practicing converting professional labeling to the “PLR” format
- Rheutopia’s labeling is fairly complex
  - Four indications
  - Associated with several serious risks (includes a boxed warning)
  - Meets the criteria for a Medication Guide
  - Administered by injection

Prototype Development Process

Reviewed:

- Scientific literature
- Current labeling practices and guidance
- Feedback - Comments and advice

Prototype Development Process

- Risk Communications Advisory Committee
  - February 26-27, 2009
- Public Workshop
  - September 24-25, 2009
- Federal Register Notice Comments
  - May 4, 2010
- Brookings Expert Workshop
  - July 21, 2010
- Part 15 Hearing
  - September 27-28, 2010

Prototypes

- Prototype A – Chunked bubbles of information
- Prototype B – Two boxed columns of information
- Prototype C - Modeled after OTC “Drug Facts” labeling

A

Drug Approved by FDA: 2005
Last Revised: May 2009

Rheutopia [Roo-TOH-pee-ah] (also known as arixalate)

Uses

- Rheumatoid arthritis in adults. Rheutopia reduces painful and swollen joints, slows joint damage, and improves mobility and the ability to do physical activities.
- Polyarticular juvenile rheumatoid arthritis in children at least 4 years old who did not have good results from other medicines. Rheutopia reduces pain, improves mobility, and decreases the number of painful joints.
- Ankylosing spondylitis. Rheutopia reduces back pain, swelling, and improves mobility.
- Plaque psoriasis in adults who may benefit from taking medicine or receiving phototherapy (using ultraviolet light). Rheutopia improves or clears up areas of skin with psoriasis.

Important Warning: Serious Infections

- Rheutopia affects the immune system. It can lower your ability to fight infections. Do not use Rheutopia if you have an active infection.
- People taking Rheutopia have gotten serious infections including tuberculosis (TB) and infections caused by viruses, fungi, or bacteria. Some people have died from these infections.

Tell Your Doctor

Before using Rheutopia, tell your doctor if you:

- have an infection, are being treated for an infection, or think you have an infection (such as a cold, flu or skin infection).
- have TB or have been near someone who has TB. You may be tested and treated for TB.
- have any nervous system or heart problems.
- have lived in or traveled to other countries. There is more risk for getting TB or other infections in certain countries.
- have been recently been vaccinated or are scheduled to receive a vaccination (including a flu shot). You should not get a vaccination while taking Rheutopia.
- are taking the medicine Kineret (anakinra). The risk of serious infections increases when used with Rheutopia.

Call Your Doctor

Stop using Rheutopia and tell your doctor right away if you develop:

- Fever, cough, flu-like symptoms, skin infection (red, warm, painful skin or open sores). These can be symptoms of a serious infection.
- Numbness, tingling, weakness, vision problems, or dizziness. Symptoms of nervous system diseases, like multiple sclerosis, may develop or get worse.
- Chills, swollen lymph nodes, night sweats, fever, or weight loss. You may have a higher chance of getting lymph node cancer.
- Bruising, bleeding, and pale skin. Your body may not make enough blood cells to fight infection or to help stop bleeding.
- Shortness of breath, swelling of ankles or feet, or sudden weight gain. These are symptoms of heart failure that may develop or get worse.
- Chest discomfort or pain, shortness of breath, joint pain or a rash on your cheeks or arms. These may be symptoms of an immune reaction with lupus-like syndrome.

Common Side Effects

- Redness, rash, swelling, itching or bruising where the shot was given.
- Headache
- Runny nose

Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

Directions for Use

- Rheutopia is an injection (shot). Do not use Rheutopia until your doctor has shown you how to give a shot.
- Store Rheutopia in the refrigerator. Do not shake or freeze.
- If you forget to take a dose, take it as soon as you remember. Take your next dose at your regularly scheduled time.
- Your doctor will tell you how often to use Rheutopia. Do not use Rheutopia more often than prescribed.

B

Drug Approved by FDA: 2005
Last Revised: May 2009

Rheutopia [Roo-TOH-pee-ah] (also known as arixalate)	
Uses	• Rheumatoid arthritis in adults. Rheutopia reduces painful and swollen joints, slows joint damage, and improves mobility and the ability to do physical activities. • Polyarticular juvenile rheumatoid arthritis in children at least 4 years old who did not have good results from other medicines. Rheutopia reduces pain, improves mobility, and decreases the number of painful joints. • Ankylosing spondylitis. Rheutopia reduces back pain, swelling, and improves mobility. • Plaque psoriasis in adults who may benefit from taking medicine or receiving phototherapy (using ultraviolet light). Rheutopia improves or clears up areas of skin with psoriasis.
Important Warning: Serious Infections	• Rheutopia affects the immune system. It can lower your ability to fight infections. Do not use Rheutopia if you have an active infection. • People taking Rheutopia have gotten serious infections including tuberculosis (TB) and infections caused by viruses, fungi, or bacteria. Some people have died from these infections.
Tell Your Doctor	**Before using Rheutopia,** tell your doctor if you: • have an infection, are being treated for an infection, or think you have an infection (such as a cold, flu or skin infection). • have TB or have been near someone who has TB. • have any nervous system or heart problems. • have lived in or traveled to other countries. • have recently been vaccinated or are scheduled to receive a vaccination (including a flu shot). • are taking the medicine Kineret (anakinra).
Call Your Doctor	**Stop using Rheutopia** and tell your doctor right away if you develop: • Fever, cough, flu-like symptoms, a skin infection (red, warm, painful skin or open sores). • Numbness, tingling, weakness, vision problems, or dizziness. • Chills, swollen lymph nodes, night sweats, fever, or weight loss. • Bruising, bleeding, and pale skin. • Shortness of breath, swelling of ankles or feet, or sudden weight gain. • Chest discomfort or pain, shortness of breath, joint pain or a rash on your cheeks or arms.
Common Side Effects	• Redness, rash, swelling, itching or bruising where the shot was given. • Headache • Runny nose Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.
Directions for Use	• Rheutopia is an injection (shot). Do not use Rheutopia until your doctor has shown you how to give a shot. • Store Rheutopia in the refrigerator. Do not shake or freeze. • If you forget to take a dose, take it as soon as you remember. Take your next dose at your regularly scheduled time. • Your doctor will tell you how often to use Rheutopia. Do not use Rheutopia more often than prescribed.

Drug Approved by FDA: 2005
Last Revised: May 2009

C

Rheutopia [Roo-TOH-pee-ah] (also known as arixalate)

Uses

- Rheumatoid arthritis in adults. Rheutopia reduces painful and swollen joints, slows joint damage, and improves mobility and the ability to do physical activities.
- Polyarticular juvenile rheumatoid arthritis in children at least 4 years old who did not have good results from other medicines. Rheutopia reduces pain, improves mobility, and decreases the number of painful joints.
- Ankylosing spondylitis. Rheutopia reduces back pain, swelling, and improves mobility.
- Plaque psoriasis in adults who may benefit from taking medicine or receiving phototherapy (using ultraviolet light). Rheutopia improves or clears up areas of skin with psoriasis.

Important Warning: Serious Infections

Rheutopia affects the immune system. It can lower your ability to fight infections. Do not use Rheutopia if you have an active infection. People taking Rheutopia have gotten serious infections including tuberculosis (TB) and infections caused by viruses, fungi, or bacteria. Some people have died from these infections.

Tell your doctor before using if you

- have an infection, are being treated for an infection, or think you have an infection (such as a cold, flu or skin infection).
- have TB or have been near someone who has TB.
- have any nervous system or heart problems.
- have lived in or traveled to other countries.
- have recently been vaccinated or are scheduled to receive a vaccination (including a flu shot).
- are taking the medicine Kineret (anakinra).

Stop use and call your doctor right away if you develop

- Fever, cough, flu-like symptoms, a skin infection (red, warm, painful skin or open sores).
- Numbness, tingling, weakness, vision problems, or dizziness.
- Chills, swollen lymph nodes, night sweats, fever, or weight loss.
- Bruising, bleeding, and pale skin.
- Shortness of breath, swelling of ankles or feet, or sudden weight gain.
- Chest discomfort or pain, shortness of breath, joint pain or a rash on your cheeks or arms.

Common side effects

- redness, rash, swelling, itching, or bruising where the shot was given.
- headache
- runny nose

Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

Directions for use

- Rheutopia is an injection (shot). Do not use Rheutopia until your doctor has shown you how to give a shot.
- Store Rheutopia in the refrigerator. Do not shake or freeze.
- If you forget to take a dose, take it as soon as you remember. Take your next dose at your regularly scheduled time.
- Your doctor will tell you how often to use Rheutopia. Do not use Rheutopia more often than prescribed.

Research Questions

- How does format affect comprehension, risk perception, and processing?
- How does the order of information affect risk perceptions and preference?
  - Warning 1st: Black Boxed Warning After "Uses"
  - Directions 1st: Directions After "Uses"

(Only Warning 1st Prototypes shown for purposes of this presentation)

Research Design

Phase I: Qualitative Interviews

- One-on-one
- Face-to-face
- Conducted with members of the following groups:
  - Low literacy and chronic illnesses
  - Have RA or another indication of Rheutopia
  - General population

Expected N = 90

Research Design

Phase I: Purpose

- To obtain in-depth feedback from important patient groups
- To make sure we do not overlook problems/issues
- To determine “minor” details such as font

Research Design

Phase II: Quantitative Experiment

- Random assignment to conditions
- Each person will see only one version
- Administered via Internet

Expected N = 1,300

Research Design

Phase II: Quantitative Experiment

3 x 2 + 1 + 1 design

Research Design

Phase II: Quantitative Experiment

3 x 2

	Format		
Order	Bubbles	Columns	OTC
Warning 1st	n = 150	n = 150	n = 150
Directions 1st	n = 150	n = 150	n = 150

Research Design

Phase II: Quantitative Experiment

+ 1

Control (Med Guide) — n = 200

+ 1

Viewing Form (Hard Copy) — n = 200

Sample

- Diagnosed with rheumatoid arthritis, ankylosing spondylitis, or plaque psoriasis
- At least 30% of sample will read at or below 8th grade level
- Most will receive prototype embedded within web program

  (except for Hard Copy group)

Research

Dependent Variables

- Comprehension of information
- Perceived risk
- Self-reported ease of understanding
- Time spent on comprehension measures

Research

Example Questions

- **Open-ended**: What does Rheutopia treat?
- **Yes/no**: should you tell your doctor that you recently lived in France (yes)
- **Application**: Jack missed his dose of Rheutopia – what should he do

(multiple choice)

Timeline

- **60-day Public Comment**: closed July 4, 2010
- **30-day Public Comment**: expected by November 5, 2010
- **OMB Approval**: expected by June, 2011
- **Final Data**: expected by January 2012

Session II: Patient Perspectives – Making PMI More Effective

- Doris Peter, Consumer Reports Health Ratings Center
- Nancy Hughes, National Health Council
- Vanessa Cajina, California Immigrant Policy Center
- N. Lee Rucker, AARP Public Policy Institute
- James Heywood, PatientsLikeMe and ALS Therapy Development Institute