



Everything That Rises Must Converge

***The Co-Development of Genome-Based Therapeutics and  
Companion Diagnostic Tests.***

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# Fryback and Thornbury --1991

- Level 1: Technical efficacy - *analytical validity*
- Level 2: Diagnostic accuracy efficacy - *clinical validity*
- Level 3: Diagnostic thinking efficacy
- Level 4: Therapeutic efficacy
- Level 5: Patient outcome efficacy - *clinical utility*
- Level 6: Societal efficacy

# Current System

- CLIA - Analytical validity and quality assurance
- FDA - Analytical and clinical validity
- Third party payers and others - Clinical utility

# Modest Proposal

- Let these entities do their jobs

# CLIA

- Let CLIA regulate labs to assure a quality operation and to allow for a sampling of the evidence that analytically valid systems are in place
- Don't try to turn a well functioning sedan into a double Decker bus

# FDA

- Let FDA regulate diagnostic medical devices
- Regulation should be risk based and not determined by the business model used to bring the test to market
- Re-read the regulations

# Third Party Payers

- Let/encourage/demand third party payers make evidence based decisions
- Cost-consciousness, cost-effectiveness, cost containment should not be dirty words
- Make this the last word and make it loud

# Change the Conversation

- Less discussion of administrative, legal, regulatory niceties
- More discussion of how to tame the science so the niceties don't matter
- Back to basics - get science right



# Bad Science

- If the goal is to move toward evidence based medicine, we need good evidence
- A need for stakeholders to recognize an inexorable fourth R (Right drug, Right patient, Right time, Right data/information)
- It shouldn't be this hard
- Need for leadership in the design and orchestration of studies

# The Choice – W.B. Yeats

- The intellect of man is forced to choose  
perfection of the life, or of the work,  
And if it take the second must refuse  
A heavenly mansion, raging in the dark.  
When all that story's finished, what's the news?  
In luck or out the toil has left its mark:  
That old perplexity an empty purse,  
Or the day's vanity, the night's remorse.

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