LIES, DAMNED LIES AND COST-EFFECTIVENESS: OPEN-SOURCE MODELS ARE ESSENTIAL IF COST-EFFECTIVENESS ANALYSES ARE TO BE WIDELY ACCEPTED: CON VIEWPOINT

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NAVIGANT

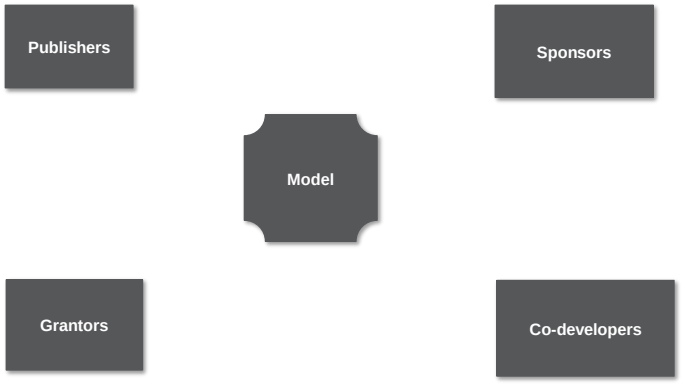
Concerns About Open CEA Models

- Intellectual property rights/payment
- Trust
- Model access (who, when, how)
- Model storage/maintenance/updating

Intellectual property rights

IP

Multiple Stakeholders (Model Access)



Proprietary vs. open access software

- Proprietary software
  - Developed and owned by single individual or entity
  - Source code is kept secret and is protected by copyright
    - License required

- "Open Source" software
  - Developed by community in collaboration
  - Source code is available, or "open", to others and they can use it without restriction, improve it, troubleshoot, build on, etc.
  - Different forms of open source licenses
    - Copyleft licenses--must also release source code
    - Share source code without charging licensing fee

IP (Trust)

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- How does one determine whom to trust with the code?
- How knowledgeable is the person who is requesting open access?
- Who even determines what is the “correct” way to use it?

Model access (who, when, how)

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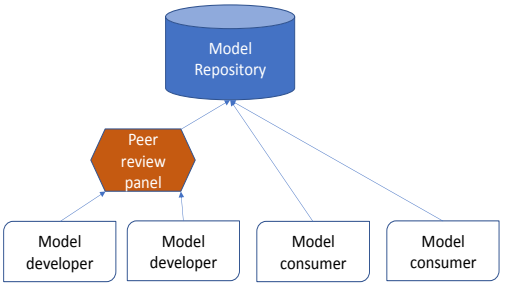
Model Access

- Who will have access?
- Terms of access
  - Free
  - Fee (fair?)
  - Short-term/long-term
  - Specific circumstances or freely available?
- Type of license
  - Individual licenses
  - Institutional
    - Seats within the institution or only individual licenses
- How do we make terms restrictive "enough" so that developer(s) feel fairly compensated, but not so onerous that use by others becomes cost-prohibitive?

Model storage/maintenance/updating



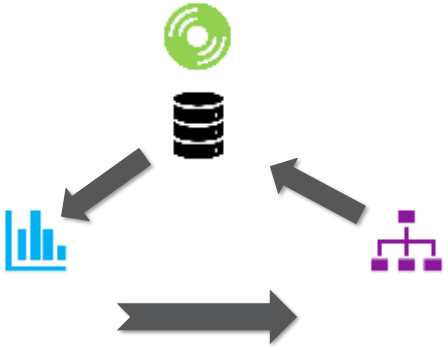
Concerns (Access)

- Logistics for use/modification/updating
- Pharmaceutical company “work for hire” owned by the sponsor
  - How will the sponsor be fairly compensated for making an updated version available to the “competition”?
- Where will the model be stored—on the developer’s secure server and accessed remotely or downloadable to the user’s computer?
- How will the model be accessed by manuscript reviewers?

Concerns (Updating)

- How will user update the model with new or other data sources:
  - Upload new data to the developer’s machine and have the developer implement the model with the new data
  - Download model and implement on their own with no requirement to confer with the developer on how the model is being modified

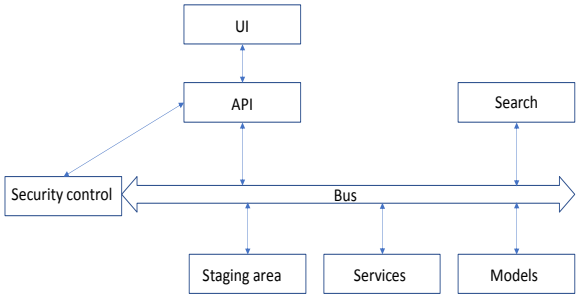
Federated Data Models



Concerns (Updating)

- Incentive for developer to maintain/keep current the model they have “posted”?
- Control of model “versioning”?
- If proprietary software, e.g., TreeAge, is used to develop the model, how will it be “opened” and modified without software license, which can be expensive

Marketplace UI



Concerns

- Who will **adjudicate** these copyright and other use issues in a "model marketplace"?
  - Panel or individuals?
    - Panel composition (central body and/or independent expert committee)
    - Partially or fully automated
- How will people be encouraged to **contribute** to the marketplace?
  Data sharing of any type prohibitive in the past due to lack of:
  - Human and computational resources
  - Bioinformatics expertise
  - Legal agreements that enable sharing
- **Initiation of request** to use an existing model
  - Objectives, methods and terms sought by the user
  - Who will evaluate this request?
  - How will we make sure that neither side—the potential user nor the evaluator(s)—is biased towards the request?

Cultural Shift Necessary

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- Who will lead this shift from proprietary to open models?
- Who will fund this shift?
- How will this be maintained in the long-term?
- How can this be encouraged in a culture where secrecy, competition and one-ups-man-ship has been the norm from time immemorial?

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PRESS RELEASE
March 30, 2018

For Immediate Release
Contact: David Whitrap
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Institute for Clinical and Economic Review Announces New Program to Make Available Draft Executable Economic Models During Drug Assessment Review Process

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Dr. Arnold is a pioneer in the development and use of HEOR methods in comparative effectiveness research. During her 20-plus-year career in the field, she has worked with such Fortune 500 companies as Pfizer, Merck, Lilly, Abbott, Bristol-Myers Squibb, Celgene, Novartis and Sanofi, as well as smaller biotechnology companies, such as BioMarin, Bracco Diagnostics and Trius Therapeutics, to develop and execute innovative methods of generating and presenting evidence to further adoption of their therapeutic offerings. As such, Dr. Arnold has worked in diverse therapeutic areas, including cardiology, oncology, nephrology, dermatology, pulmonology, endocrinology, infectious diseases and rare (genetically-based) diseases. Dr. Arnold was most recently VP, HEOR, Quorum Consulting, Inc.; Principal, IMS Health; President and Co-Founder of Pharmacon International, Inc. Center for Health Outcomes Excellence and President & CEO, Arnold Consultancy & Technology LLC, headquartered in New York City.

As Vice President of HEOR, Dr. Arnold oversaw outcomes research and designs custom data-collection structures for outcomes and health economics research for pharmaceutical and federal government programs. Her special interest in evidence-based health derives from her research in using technology to collect and/or model real-world data for use in rational decision-making by healthcare practitioners and policy makers. Dr. Arnold is also Adjunct Associate Professor, Master of Public Health program, Department of Environmental Health and Preventive Medicine at the Icahn School School of Medicine at Mount Sinai, New York, NY, where she has developed and teaches the pharmacoeconomics coursework. Dr. Arnold is a founding member of the International Society for Pharmacoeconomics and Outcomes Research (ISPOR) and is currently Chair of the ISPOR Distance Learning Committee. She has authored and co-authored numerous articles and book chapters in the areas of pharmacology, pharmacoeconomics and cost containment strategies. She is also a recipient and reviewer of U.S. National Institutes of Health (NIH) Small Business Innovation Research (SBIR) grants, as well as a reviewer for Patient-Centered Outcomes Research Institute (PCORI) grant applications and numerous medical journals.

Dr. Arnold completed her undergraduate training at the University of Maryland and received her Doctor of Pharmacy degree from the University of Southern California in Los Angeles. She also completed a one-year post-doctoral residency at University Hospital in San Diego/University of California at San Francisco School of Pharmacy