

Precision Medicine: Lecture 17

Overview Discussion

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Outline

Overview of Topics

Challenges

Next Steps

Causal Inference and Machine Learning

- ▶ Causal inference is central to population-based precision medicine and precision health
- ▶ We discussed a number of relevant machine learning techniques:
 - ▶ Traditional statistical models
 - ▶ Support vector machines
 - ▶ Random forests
 - ▶ Deep learning and related techniques
 - ▶ Gaussian processes

Single Decision Setting

- ▶ Methods
 - ▶ Regression and policy approaches
 - ▶ Double robust methods
 - ▶ Outcome weighted learning
- ▶ Actions
 - ▶ Binary and multiple (nominal)
 - ▶ Continuous and more complex
- ▶ Outcomes
 - ▶ Binary or continuous
 - ▶ Right censored
 - ▶ Multiple outcomes
- ▶ Study designs
 - ▶ Randomized
 - ▶ Observational
 - ▶ Adaptive

Multi Decision Setting: Finite Horizon

- ▶ Methods
 - ▶ Reinforcement learning (off-policy)
 - ▶ Q-learning
 - ▶ Outcome weighted learning
- ▶ Actions
 - ▶ Binary and multiple (nominal)
 - ▶ Continuous and more complex (not discussed)
- ▶ Outcomes
 - ▶ Binary or continuous
 - ▶ Right censored
 - ▶ Multiple outcomes (not discussed)
- ▶ Study designs
 - ▶ SMART
 - ▶ Observational
 - ▶ Hybrid, adaptive

Multi Decision Setting: Indefinite Horizon

- ▶ Methods
 - ▶ Markov Decision Processes
 - ▶ Q-learning
 - ▶ V-learning
 - ▶ Generalized estimating equations
- ▶ Actions
 - ▶ Binary and multiple (nominal)
 - ▶ Continuous and more complex (not discussed)
- ▶ Outcomes
 - ▶ Binary or continuous
 - ▶ Multiple outcomes (not discussed)
- ▶ Study designs
 - ▶ Micro-randomized trials
 - ▶ Observational
 - ▶ Online estimation
 - ▶ Adaptive and continual reassessment

Other Topics

- ▶ Incorporating patient and physician preferences
- ▶ Inverse reinforcement learning
- ▶ Inferential challenges (value function inference)
- ▶ Sample size calculations
- ▶ Implementation in practice to improve health
- ▶ Other topics

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- ▶ PM is a new area and there is limited history of its use in practice
- ▶ PM is challenging to learn and hard to explain to collaborators, paper reviewers and funders
- ▶ In some settings, significant additional research, theory and software development is needed before the methods can be fully understood and implemented
- ▶ Study design in PM can be much more complex than in traditional settings
- ▶ The emphasis on discovery versus hypothesis testing can be confusing

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What Next?

- ▶ Continue learning
- ▶ Research open questions, in both applications and foundations
- ▶ Help expand reach of precision medicine and precision health:
 - ▶ Participate in biomedical collaborations
 - ▶ Help improve and implement new methods and address challenges

Relevant Technical Areas

- ▶ Optimization: use of surrogates, convexification, loss functions, gradient methods, non-convex objective functions, etc.
- ▶ Empirical processes: consistency, error bounds, rates of convergence, weak convergence, asymptotically valid inference, M-estimators, Z-estimators, functional delta method, etc.
- ▶ Semiparametric inference: efficiency and optimality of estimators, tangent sets, Hilbert spaces, infinite dimensional efficiency, inference, etc.
- ▶ Other advanced statistics topics: functional data, nonparametric estimation and inference, etc.
- ▶ Foundations of causal inference and data-driven decision support (e.g., Rémy Tuyéras, 2020, "A category theoretical argument for causal inference", arXiv)