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New Developments in Precision Medicine : Implications for Practice and Policy

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What is Precision Medicine?

"An emerging approach for disease treatment and prevention that takes into account individual variability in genes, environment, and lifestyle"
(US National Institutes of Health)

- Ability to predict more accurately which treatment and prevention strategies will work for whom allows us to (i) improve outcomes and (ii) avoid adverse effects or treatment toxicities
- Similar to "personalized medicine", but not focused on treatment regimes developed uniquely for each individual, rather on identifying which approaches will be effective for which identifiable groups of patients based on genetic, environmental, and lifestyle factors.

Pharmacogenomics

Individual's susceptibility to certain diseases or response to a treatment are sometimes linked to specific common DNA variations, or single nucleotide polymorphisms (SNPs)

Pharmacogenomics can tell us about specific genes encoding either metabolic enzymes or defective structural proteins which lead to

- The need for a higher dose to achieve a therapeutic effect
- A greater risk of side effects or more severe side effects
- Variation in effect size or likely benefit from the treatment

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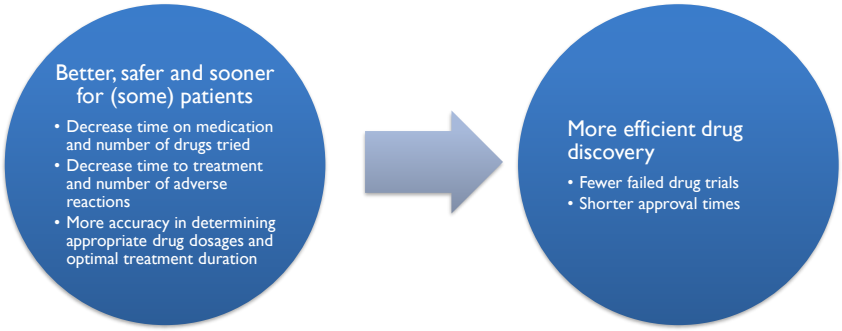
Example : Acute myeloid leukemia (AML)

- Combinations of various doses and schedules of drugs used as treatment for all) types of AMLs (of which there are many)
- Significant variation in responses, some of which are very severe adverse reactions
- Prognostic and predictive biomarkers can identify patients who could benefit from a particular treatment or those exhibiting higher risks of toxicity.

Potential for Patients And Pharma



Peril as Well…

- Field is still in early stages, unclear that most "high-tech" discoveries are sufficient to be clinically meaningful
- Need to look beyond genetics towards a systems approach, but adding biological, environmental, and behavioral data = massive conceptual and computational problem
- Cost of genetic sequencing is falling but still high
- Cost of drugs may stay high with potential for price-discrimination
- Opens up new ethical and practical considerations for practice

Ivacaftor

Can reverse disease in patients with a specific mutation in the CF gene

Fast-tracked by the FDA

Cost-effectiveness is not guaranteed

- Costs up to $300,000 a year per patient
- For patients with suitable mutation, recent evidence suggests impact equal to that of three universal (and cheaper) treatments: high-dose ibuprofen, aerosolized saline and the antibiotic azithromycin
- At present, does not help 95% of the entire CF patient population

What does the future hold?