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Big Data for the Benefit of Patients

Precision Medicine and the Challenges of Different Kinds of Data

Cologne, May 23, 2016

Friedrich von Bohlen

Definition

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‚*Personalized Medicine*‘ isn‘t new. Basically, medicine has always been ‚*personalized*‘.

It is rather synonym or ‚terminus technicus‘ for another definition used these days: ‚*Precision Medicine*‘.

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Precision Medicine

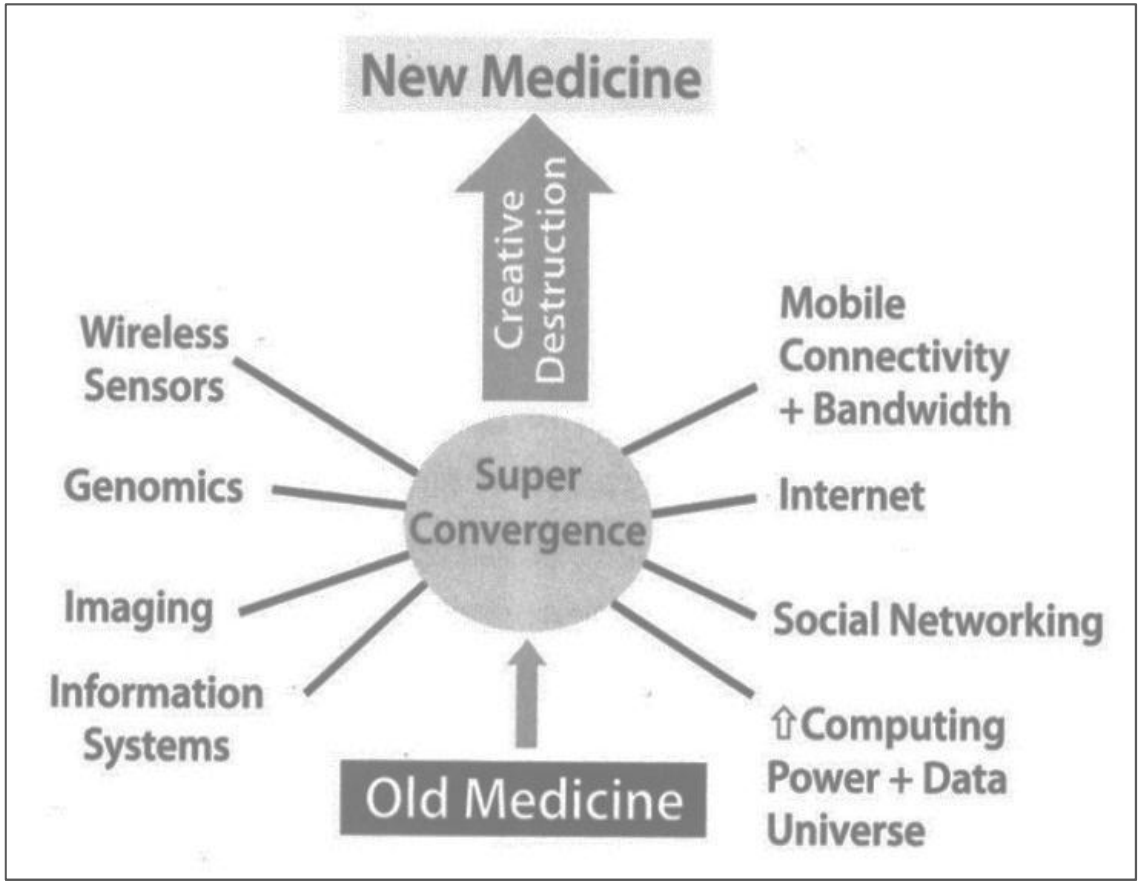
Transformation from ‚Old' to ‚New Medicine'

Molecularities

- Genome
- All other ‚-omes' in the future

IT

- Databases
- Integration
- Interpretation
- Presentation
- Networks



(Quelle: Eric Topol „The Creative Destruction of Medicine", Basic Books, New York 2012, page VII)

Situation

Precision Medicine is ‚Big Data', but…

TODAY

CURRENT DISEASE FOCUS:
Oncology (mostly)

CURRENT "-OMES":
Genome: 22,000 genes, but today mostly panels (50-500 genes)

CURRENT DRUGS:
8,000

TOMORROW

FUTURE DISEASE FOCUS:
Ca. 30,000 different diseases

FUTURE "-OMES":
Genome: 22,000 genes
Transcriptome: 120,000 transcripts
Proteome: 500,000 proteins
Metabolome: >1,000,000 metabolites

FUTURE DRUGS:
>15,000, even individual drugs

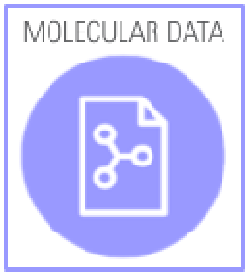
CURRENT AND FUTURE NUMBER OF PATIENS IN LONGITUDINAL TRANSLATIONAL DATABASES

<50,000 >500,000,000

$$\text{Value} \sim n^{(x+y+z)}$$

Situation

…Precision Medicine is even more an integration- and interpretation challenge



nanograms of DNA

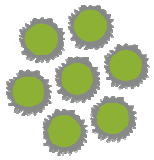
countless drug combinations and co-medications

> 8,000 approved medications

3 billion genomic positions - > 22,000 genes

complex co-morbidities, medical conditions and adverse events

> 10,000 clinical studies



Each disease is molecularly different

translational longitudinal data

> 23 Mio. scientific publications

Precision Medicine

Will irreversibly change medicine and healthcare

From an

observation- and cellulary-based, IT-supported medicine

To an

IT-centric, molecular sciences-based medicine.

From a

guideline-driven and -reimbursed medicine

To a

system-/evidence-based, more outcome-reimbursed medicine.

From a

hierarchically grown and organized medicine

To a

‚digitalized and democratized medicine‘.

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Benefits of Precision Medicine

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Individual molecularly-based diagnosis, precise efficacious and safe therapies and therapy options at every time and state of a disease.

Decision making algorithms to add and amend to human expertise and capabilities.

Pharma can develop drugs better: targeted, faster, cheaper.

Payers and all stakeholders can understand benefit/cost ratios.

Patients will live better + longer.

Specific therapy costs drop.

New reimbursment and insurance models.

Population health data to become a new currency in medicine.

Society will need to (re-)think and (re-)act.

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Benefits of Precision Medicine

For Pharma

Drug development oriented at the molecular understanding of a disease:

- **Precise stratification enabling clinical studies with smaller number of patients, shorter study times and higher success rates.**
- **Reduces high cost of failed studies and extends net patent span.**
- **Better care, improving the therapeutic spectrum and forms basis for rational and safe combination therapies.**

Better drugs and therapy combinations at lower cost.

Benefits of Precision Medicine

For Regulators

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Better understanding and control of study designs, approval-relevant information, rationales for combinations and ‚off-label' use, improved pharmacovigilance (Phase IV studies and post-approval data).

Drug safety labeling based on drug mechanism and molecular patient profile: from observation- to mechanism-based drug and therapy safety.

Dynamic, mechanism-based understanding of drug combinations requiring novel approaches for approval and regulation.

No more drug approval without understanding of mechanism of action and companion diagnostics (‚biomarker').

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Benefits of Precision Medicine

For Payers

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Better understanding of the individual value of a therapy/prescription and of combinations. Dynamic definitions will replace ‚on label' and ‚off label' use.

Benefit/cost can rationally be understood, calculated and valued.

Increasing rationally-based combination therapies and poly-pharmacies need novel pricing structures and schema.

Future: personal risk profiles allow better prevention and prophylaxis and will lead to novel reimbursment structures.

New understanding of benefit/cost of diagnosis and therapy.
Novel and differentiated reimbursment principles and schema.

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Benefits of Precision Medicine

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For Hospitals/Centers

Most likely hospitals will become ‚owners' of molecular patient data and profiles and longitudinal treatment information.

These data, together with the primary-literature world knowledge, will become the reference system for future therapies and stratifications.

These longitudinal data have their own value which will lead to novel monetization models in healthcare systems.

Not generation of data, but their conextualization and interpretation for diagnosis and therapy, and ‚supply-demand' systems of and for patient will become important and valuable in the future.

Population health data to become a new currency in medicine.
Will transform ‚direct' to ‚multi-sided' and ‚market place' models.

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Benefits of Precision Medicine

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Last but not least: The Patient

Patients/consumers will be equally well informed as doctors and payers.

Smartphones will carry health records as well as many other concomitant health- and lifestyle information important for diagnosis and treatment.

Connectivity of systems including mobile systems of patients will be the driver for ‚democratized medicine‘.

Medicine will turn into an open online medicine. Patient profiles can be used real-time for diagnosis and therapy. These data will remain basis of health and payer systems, but there will also be a parallel world in which (younger) users will have and share their data and information in social networks.

Patients become more conscious and active players for their health.
Patients live better + longer.

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Summary

"My physician prescribed a personalized therapy. Here's my DNA sequence"