

# The Future of Medicines Development and the Role of Expert Patients

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Dr. Tony Hoos

Head of Medical, Amgen Europe

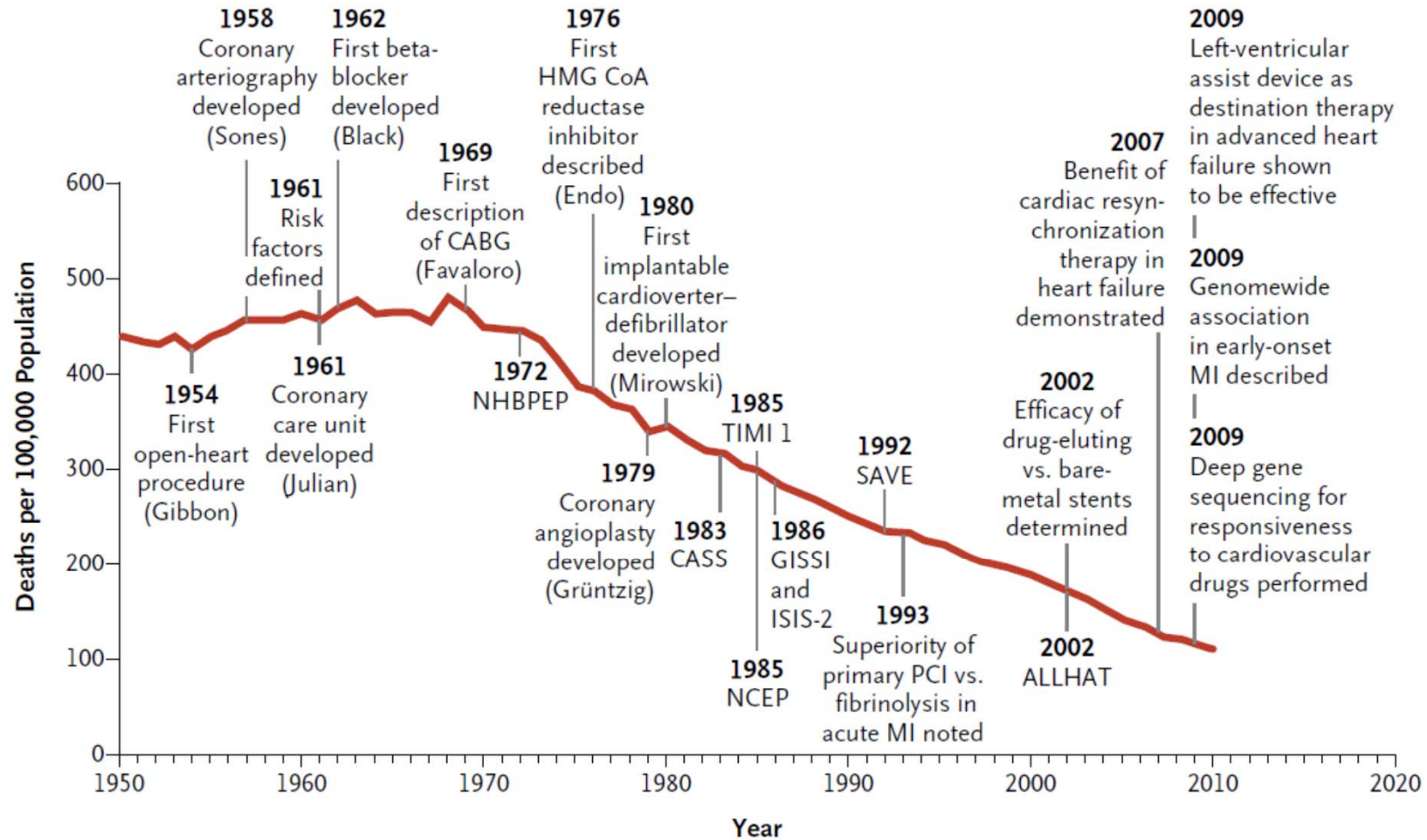
14 December 2016, Brussels, Belgium

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**Curing / treating diseases:**

**Innovation is the enabler of progress**

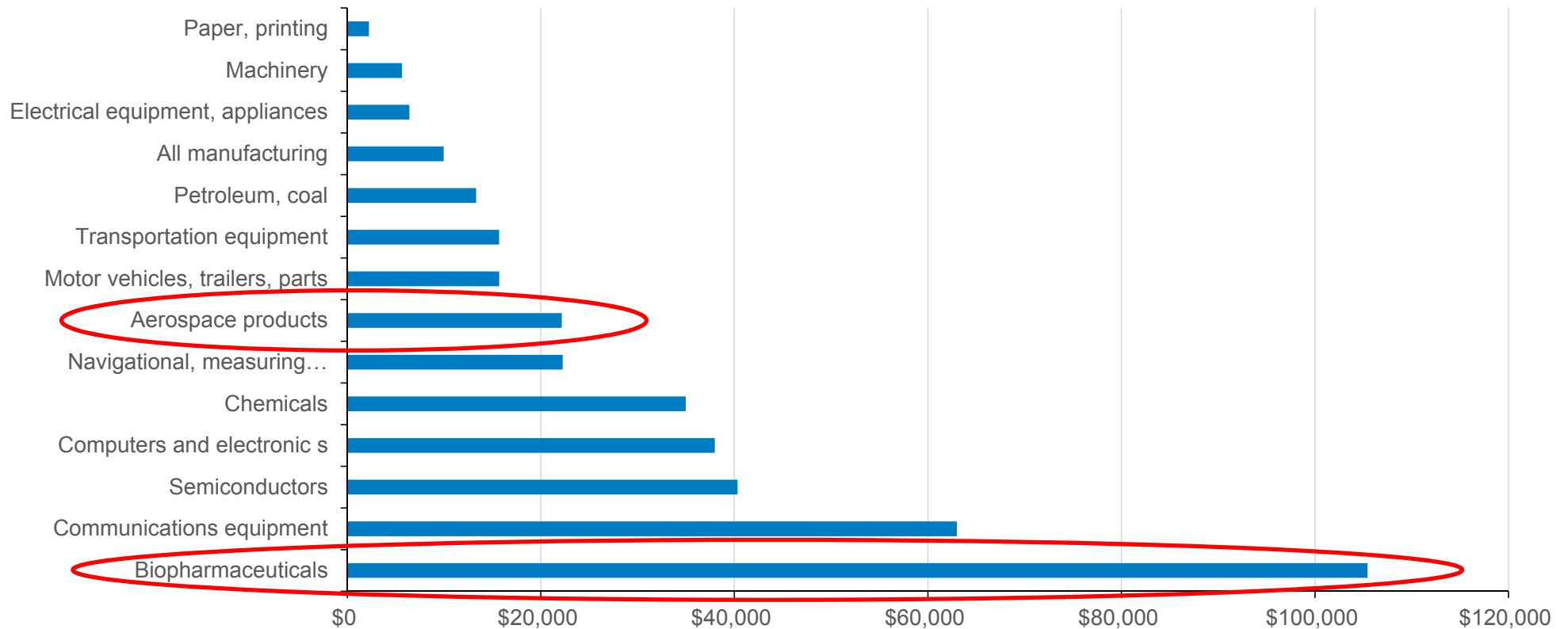
# Decline in Deaths from Cardiovascular Disease in Relation to Scientific Advances



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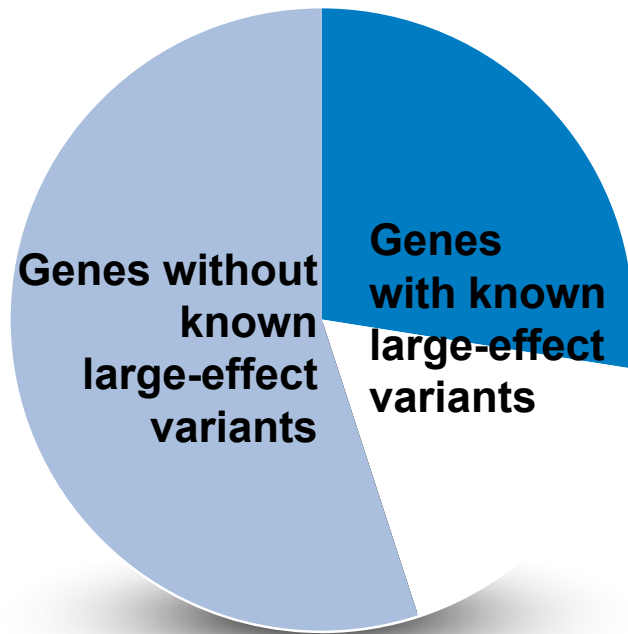
**THE ESTIMATED AVERAGE PRE-TAX  
INDUSTRY COST PER NEW  
PRESCRIPTION DRUG APPROVAL  
(INCLUSIVE OF FAILURES AND CAPITAL  
COSTS) IN 2014 WAS:  
**\$2,558 MILLION****

# Development of new medicines is expensive: R&D Expenditure per Employee 2000–2007



# Comprehensive Genetics: Evidence and Evidence of Absence

**Functionally Complete  
Catalog of Variants**



**~ 20,000 human genes in total**



**Sufficient Number of  
Clinical Observations**



**> 10,000 diseases**

# Human Genetics Can Improve...

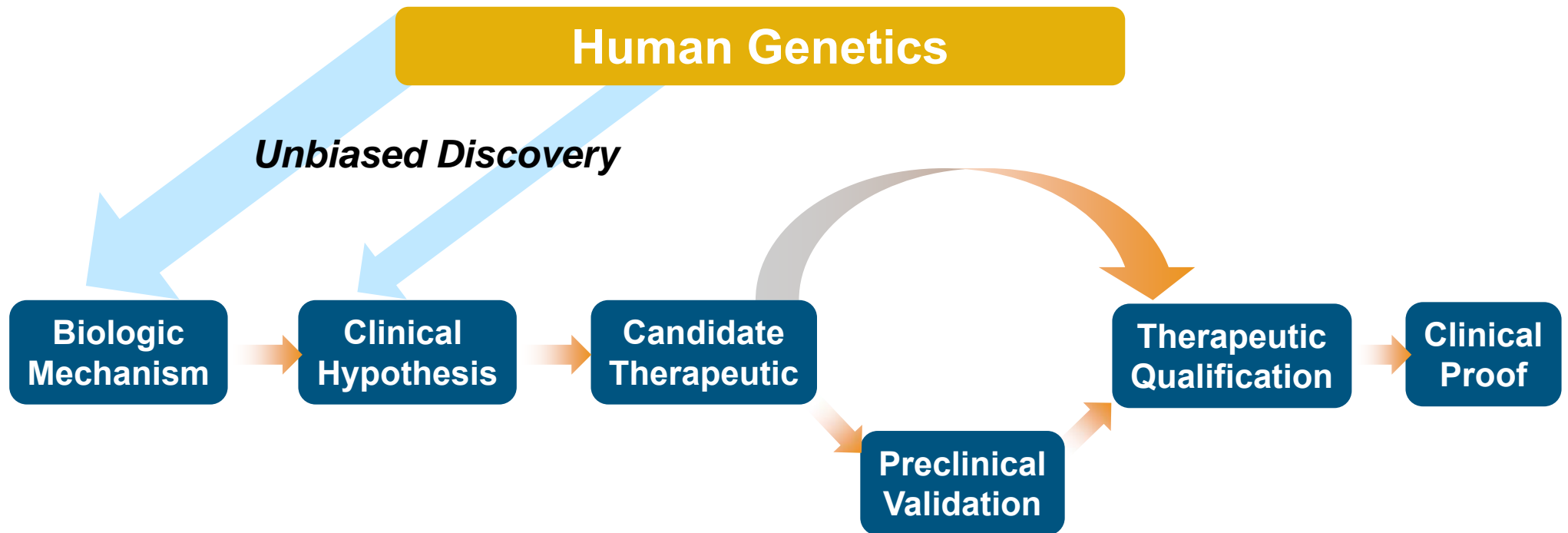
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- Clinical **success rates**
  - Observe results of genetic equivalent of a clinical trial prior to Phase 2/3 investment
- **Speed** and efficiency to market
  - Prove disease relevance of target and avoid entanglements in complex preclinical biology
  - Short-circuit common scenario of molecule searching for a disease
- **Differentiation**
  - Benefit from unique and unexpected biological insights

**Less guesswork => more conviction around  
patient and time investment**

# From Clinical Hypothesis to Clinical Proof: Impact of Genetics on Speed/Differentiation/Success Rate

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# Human Genetics – a major enabler to cure / treat diseases

## Example: Cardiovascular Diseases – LDL-C

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### Success Rate

- PCSK9 → Target with compelling data for efficacy and safety ✓

### Speed

- ASGR1 → Novel clinical hypothesis for a tractable target

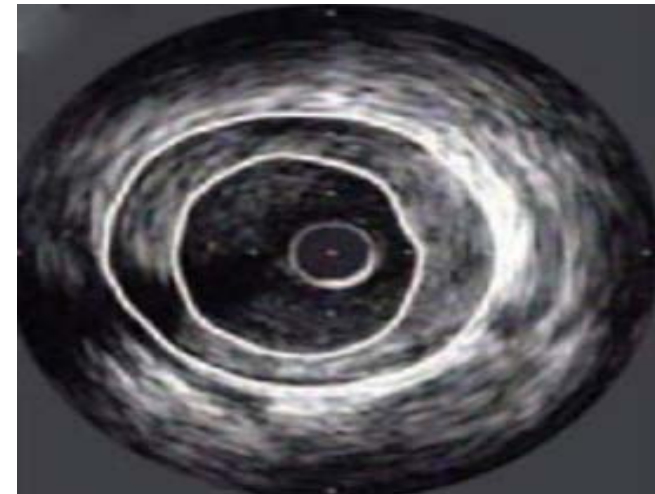
# PCSK9 inhibition LDL-C lowering and atherosclerotic plaque reduction

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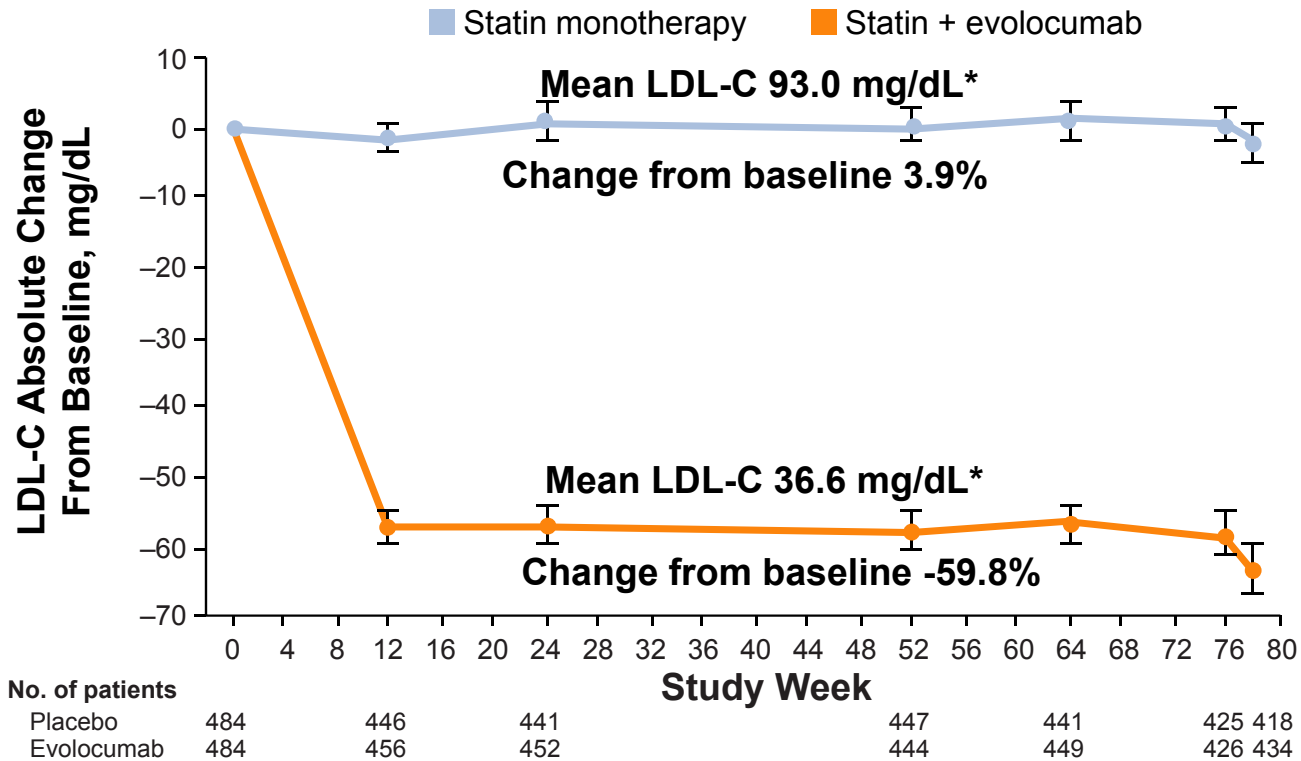
JAMA | Original Investigation

## Effect of Evolocumab on Progression of Coronary Disease in Statin-Treated Patients The GLAGOV Randomized Clinical Trial

Stephen J. Nicholls, MBBS, PhD; Rishi Puri, MBBS, PhD; Todd Anderson, MD; Christie M. Ballantyne, MD; Leslie Cho, MD; John J. P. Kastelein, MD, PhD; Wolfgang Koenig, MD; Ransi Somaratne, MD; Helina Kassahun, MD; Jingyuan Yang, PhD; Scott M. Wasserman, MD; Robert Scott, MD; Imre Ungl, MD, PhD; Jakub Podolec, MD, PhD; Antonius Oude Ophuis, MD, PhD; Jan H. Cornel, MD, PhD; Marilyn Borgman, RN, BSN; Danielle M. Brennan, MS; Steven E. Nissen, MD



# Mean Absolute Change in LDL-C

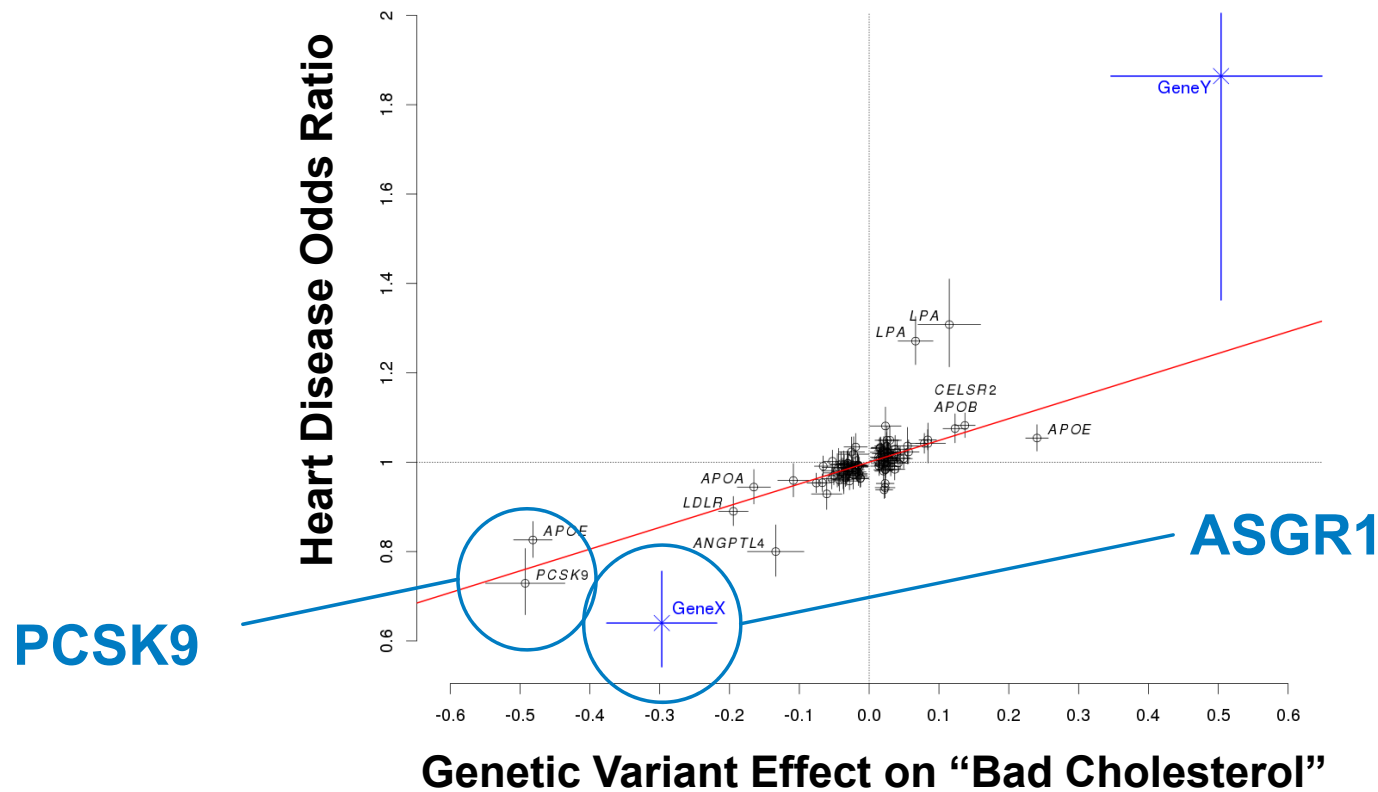


**Absolute change for evolocumab-statin group: -56.3 (-59.4 to -53.1);  $P < 0.001$**

Data shown are Mean (95% CI) \*Time-weighted LDL-C; LDL-C = low-density lipoprotein cholesterol  
 Nicholls SJ, et al. *JAMA*. [published online ahead of print November 15, 2016]. doi: 10.1001/jama.2016.16951.  
 Nissen SE, et al. *American Heart Association Scientific Sessions*, Nov 12 - 16, 2016,  
 New Orleans, Louisiana. Oral Presentation.

# Genetics Influence on Amgen Pipeline—ASGR1 Gene Example

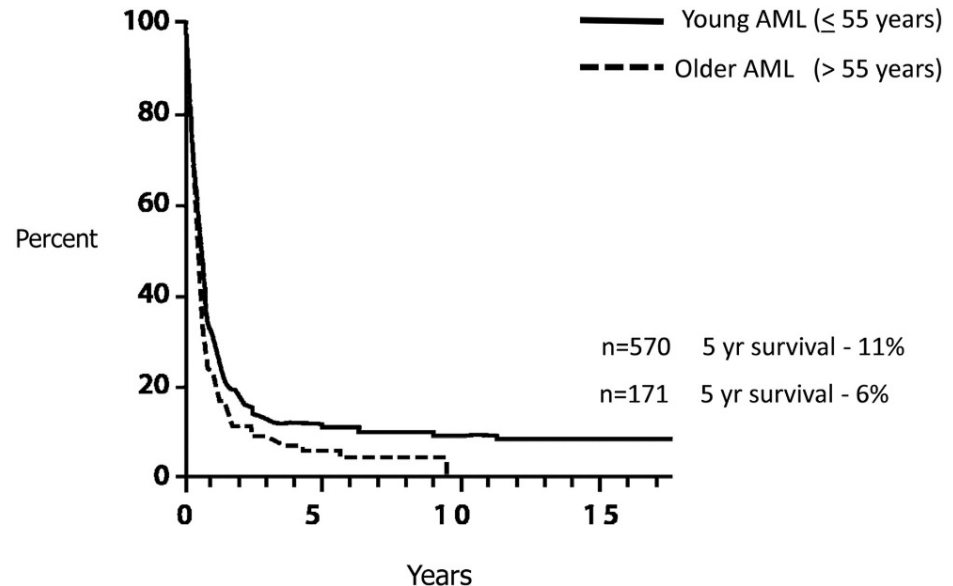
## Clinical Hypothesis: Block “ASGR1” to Treat Cardiovascular Disease



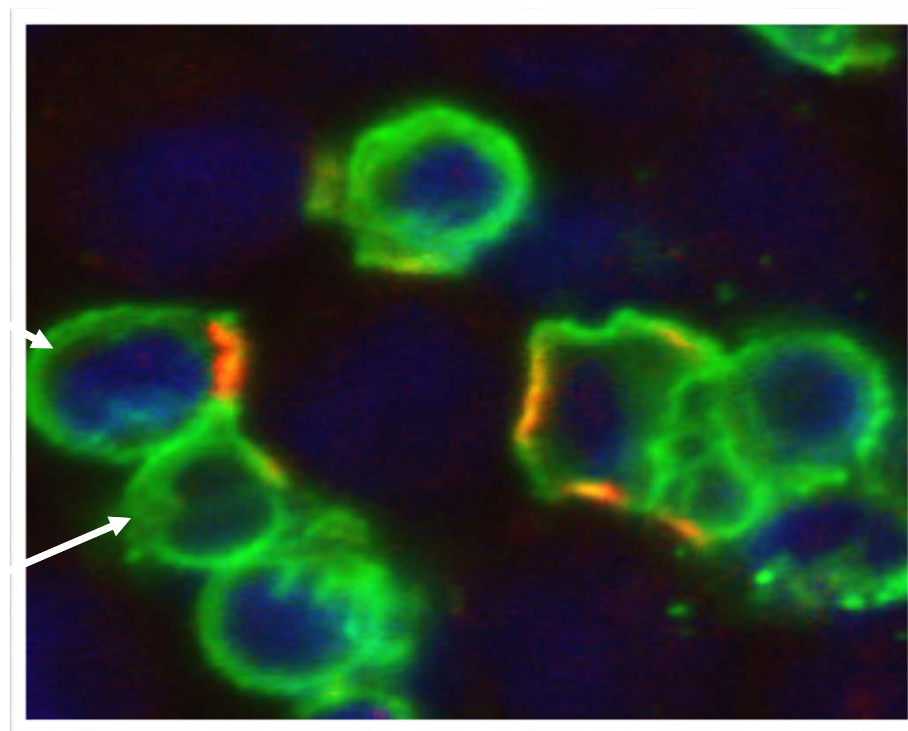
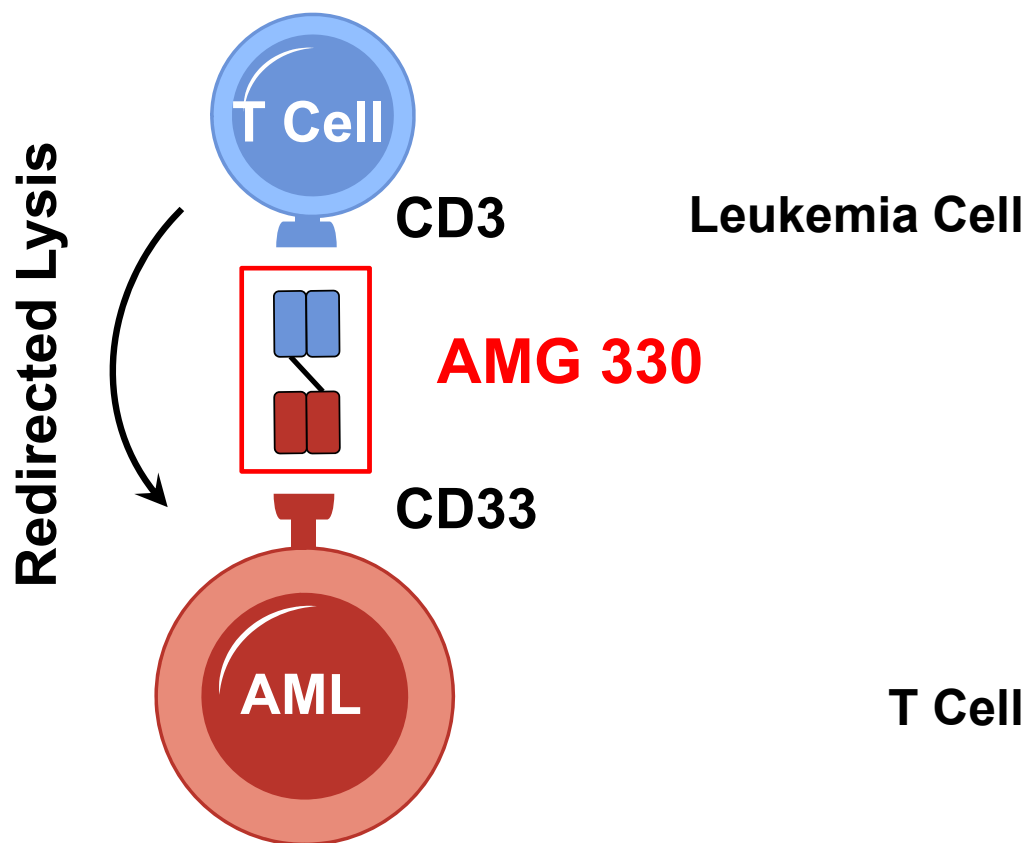
# There remains a very high unmet medical need in Acute Myeloid Leukaemia

- No standard chemotherapeutic regimen exists for the treatment of patients with relapsed or refractory acute myeloid leukaemia (AML)
- Many patients do not tolerate chemotherapy regimens
- Elderly patients and those with comorbidities and poor performance status are especially challenging to treat with existing, approved therapies
- An anti-CD33 Antibody Drug conjugate (Gemtuzumab ozogamicin), has established Proof-of-Concept for CD33 therapeutic targeting in AML

Survival From Relapse By Age



# Example Immuno Oncology – bi specific antibodies: AMG 330 (a CD33-Specific BiTE<sup>®</sup> Antibody) that kills AML (acute myeloid leukemia) Cells

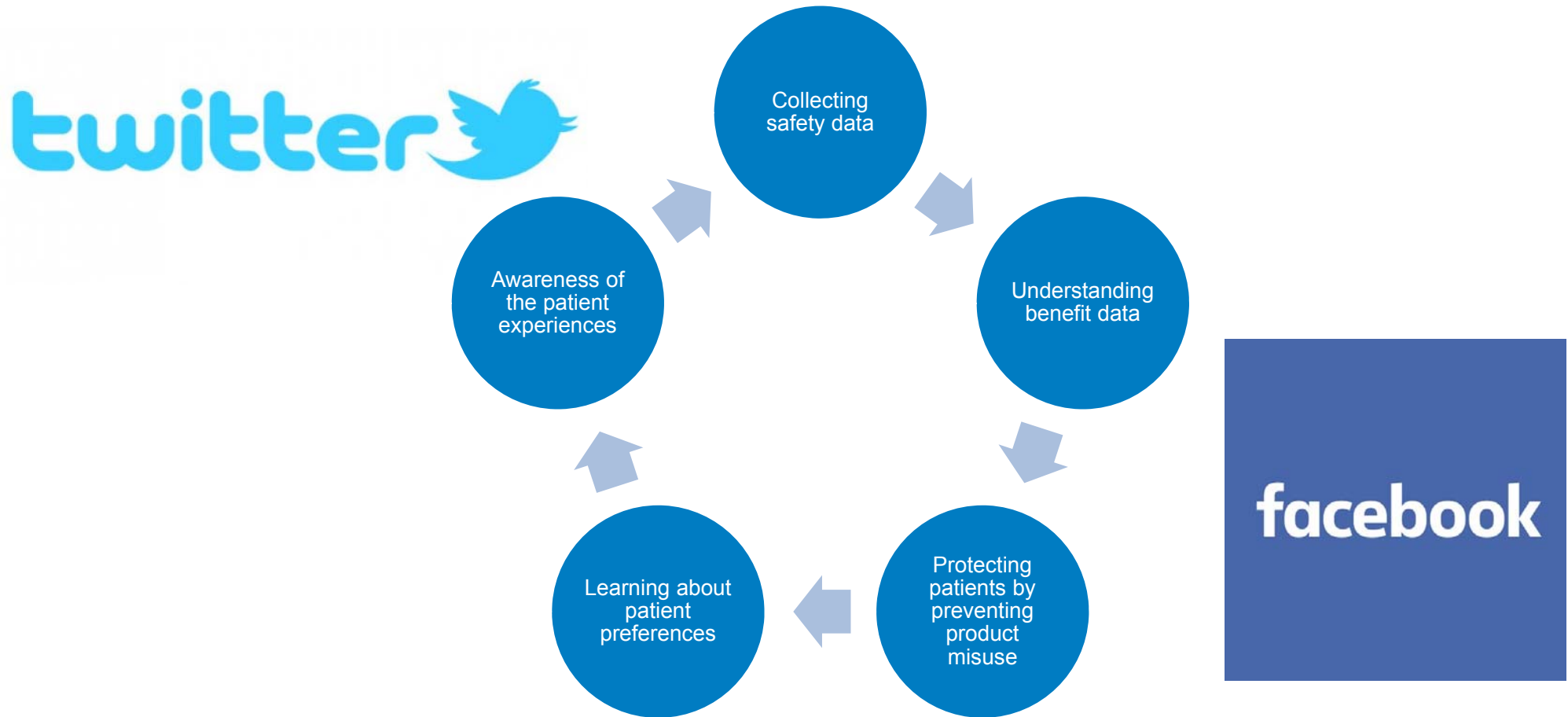


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# **CHANGING THE WAY HEALTHCARE CONSUMERS BUY AND USE HEALTHCARE**

# Using Social Media to Better Understand the Patient Journey and Patients' Needs

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# The future of diagnostics

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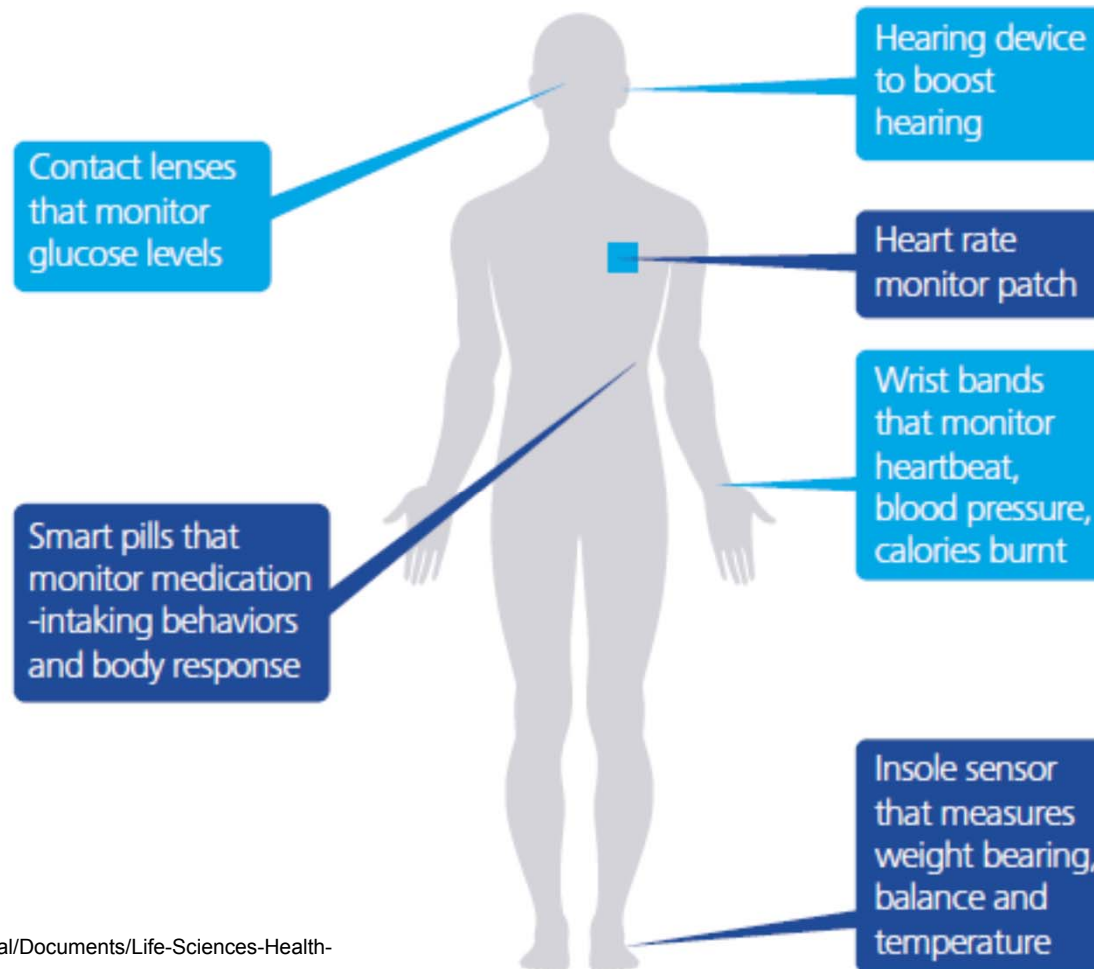
## Medical tricorders and portable diagnostics

- The fictional medical tricorder from *Star Trek* may soon to be a reality
- Diagnostic procedures are shifting towards devices that are portable and able to be performed from home
- Medical mobile applications will be prescribed with patient customisation with the smartphone likely to be key to the future of medicine
- Qualcomm is hosting a contest called Xprize (<http://tricorder.xprize.org/>) for 7 final teams to develop a Tricorder medical device

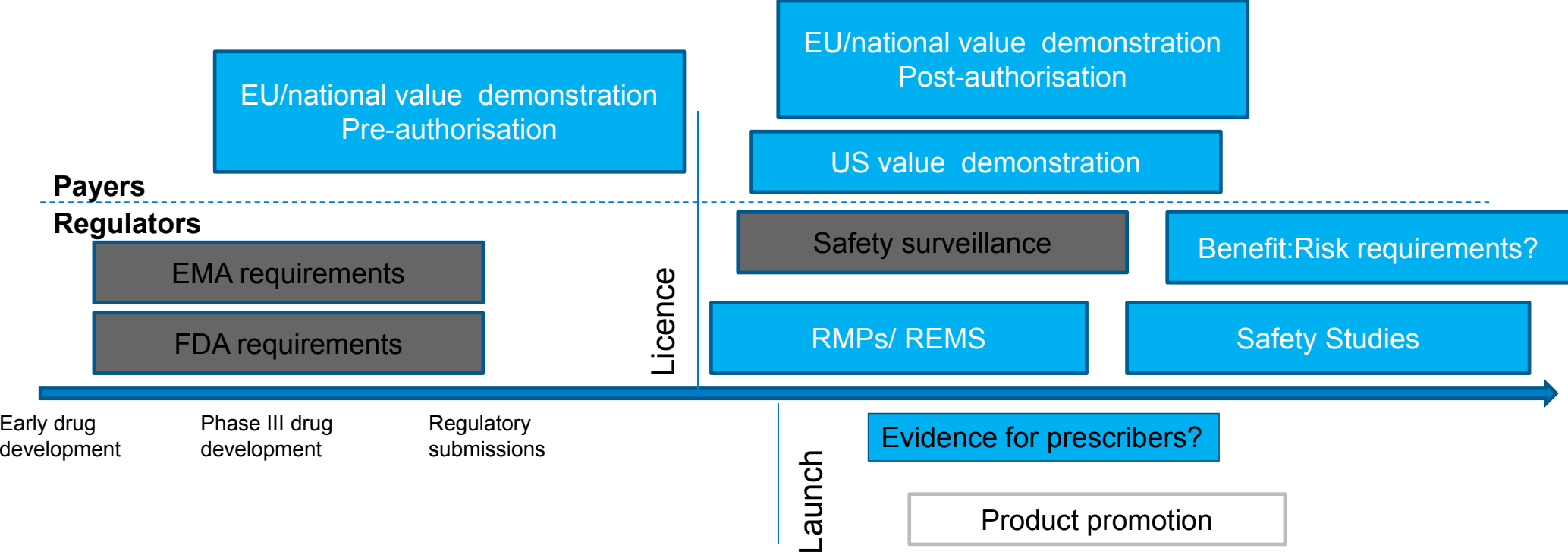


# Technology moving healthcare out of the hospital into the patient's home

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# How much data do we need to offer a medicine to patients?



EMA: European Medicines Agency -- FDA: Food and Drug Administration -- RMP: Risk Management Plan -- REMS: Risk Evaluation and Mitigation Strategies

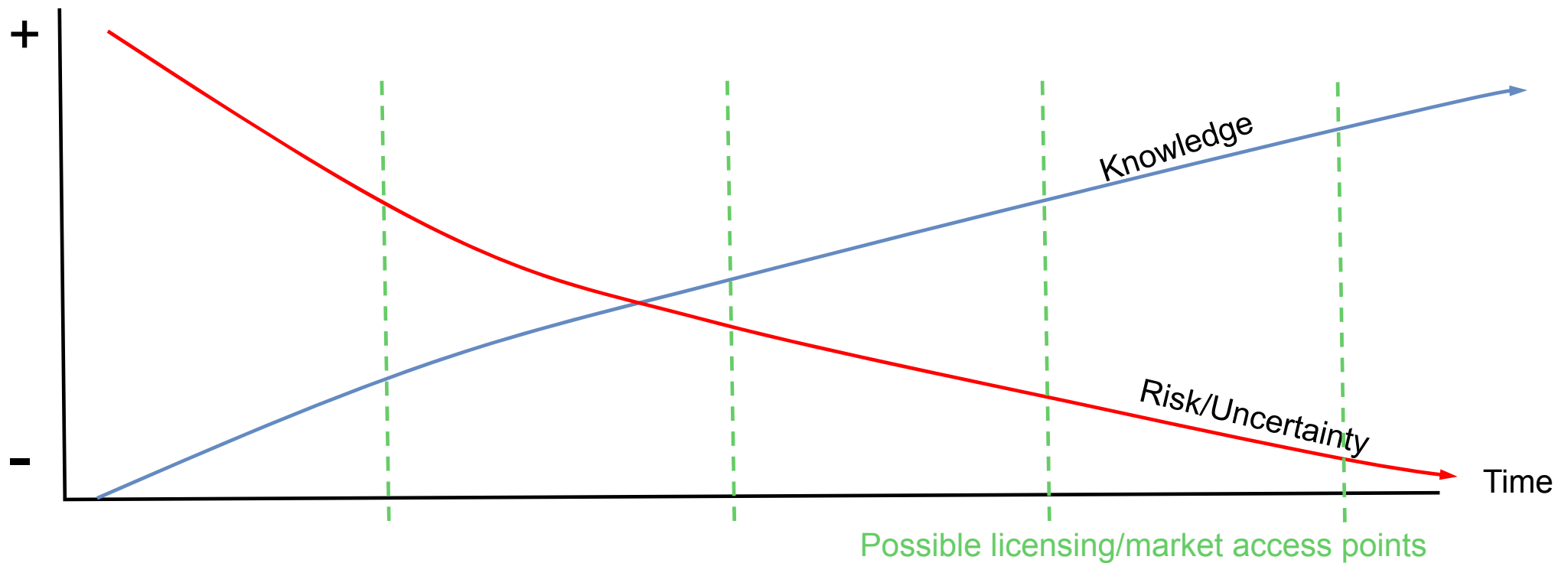


# We will have to manage complex patient data...


Figure 1 – Future types of patient data



# What is our tolerance for risk and uncertainty?



**Stakeholders need to agree on acceptable level of risk/uncertainty and benefit/risk**

A dramatic background image of a stormy sea with dark, heavy clouds and bright lightning bolts striking down. The water is dark blue with white foam from the waves.

**High cost pressures on  
health care systems**

**Current model non sustainable**

**Benefit – risk assessments**

**Value demonstration**

**Patient Centric  
Models as an  
answer?**

**Big Data**

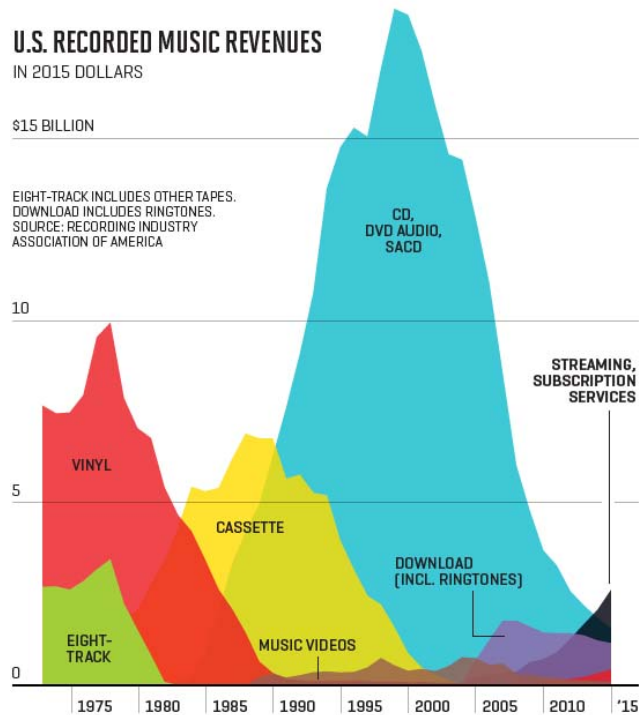
**Trust & Transparency**

**Players move in isolation –  
multi stakeholder  
alignment?!**

**New Societal  
contract required?**

# Disruptive Innovation Explained

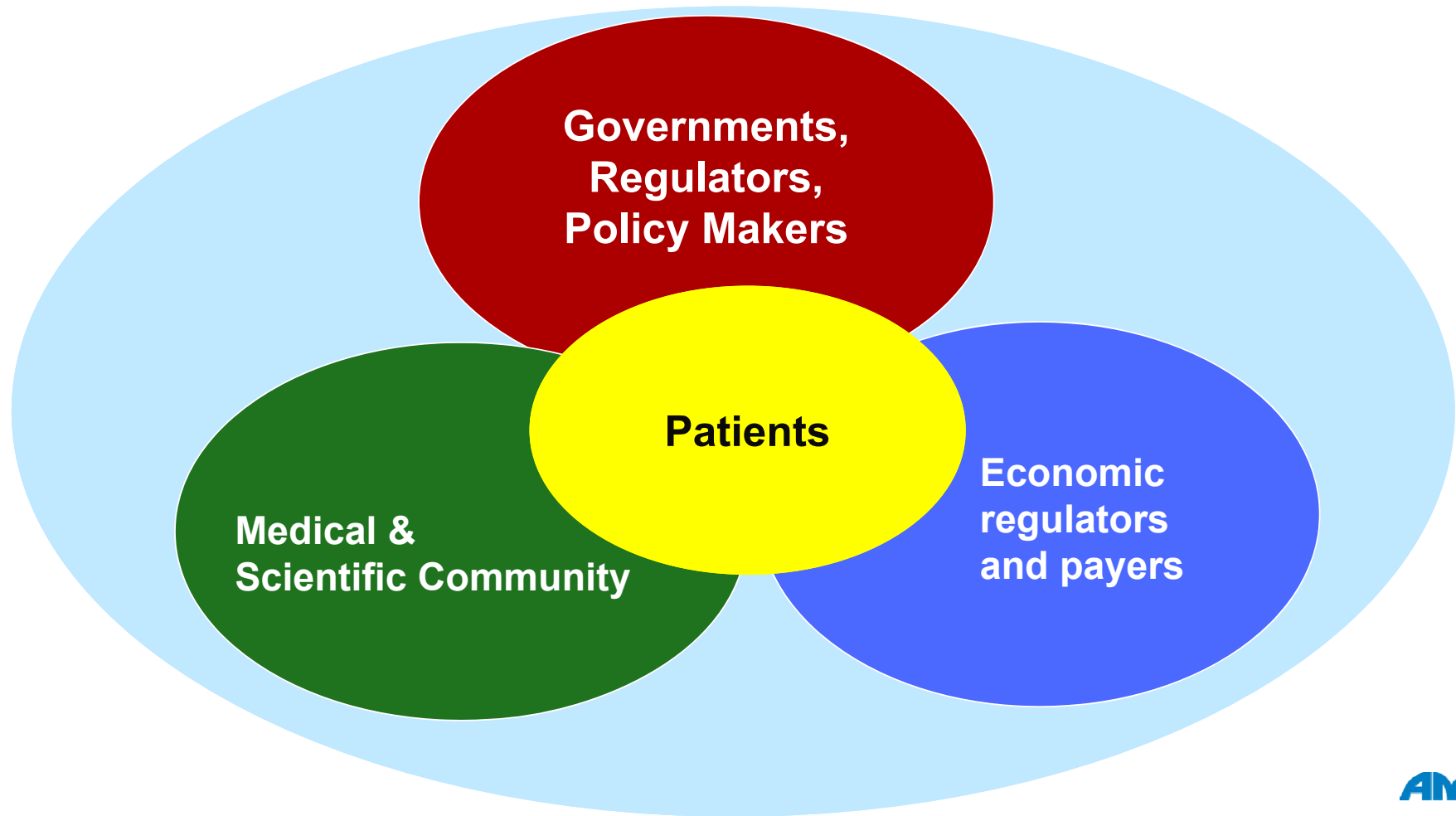
Innovation transforms an existing market or sector by introducing simplicity, convenience, accessibility, and affordability where complication and high cost are the status quo = COMPLETELY REDEFINES THE INDUSTRY



 <b>UBER</b> World's largest taxi company Owns NO <del>Taxis</del>	 <b>airbnb</b> World's largest Accommodation provider Owns NO <del>Real estate</del>	 World's largest Phone companies Owns NO <del>Telco infra</del>	 <b>Alibaba Group</b> World's most Valuable retailer Owns NO <del>Inventory</del>
 <b>facebook.</b> Most popular Media owner Owns NO <del>Content</del>	 <b>SocietyOne</b> World's fastest Growing bank Owns NO <del>Actual money</del>	 <b>NETFLIX</b> World's largest movie house Owns NO <del>Cinemas</del>	 <b>Apple Google</b> World's largest Software vendors Owns NO <del>Apps</del>

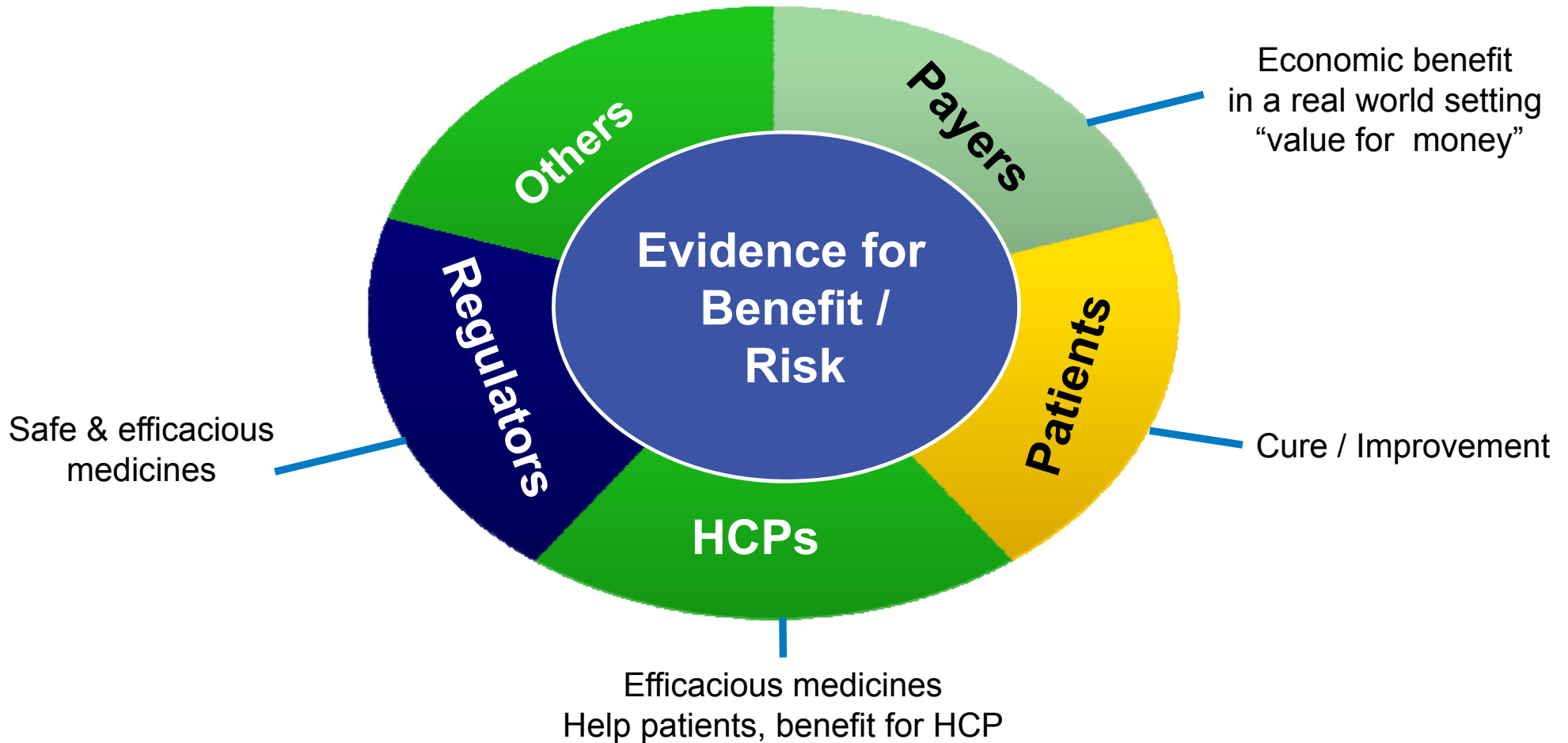
**We all serve patients & society**  
**All stakeholders must work together to change the system**

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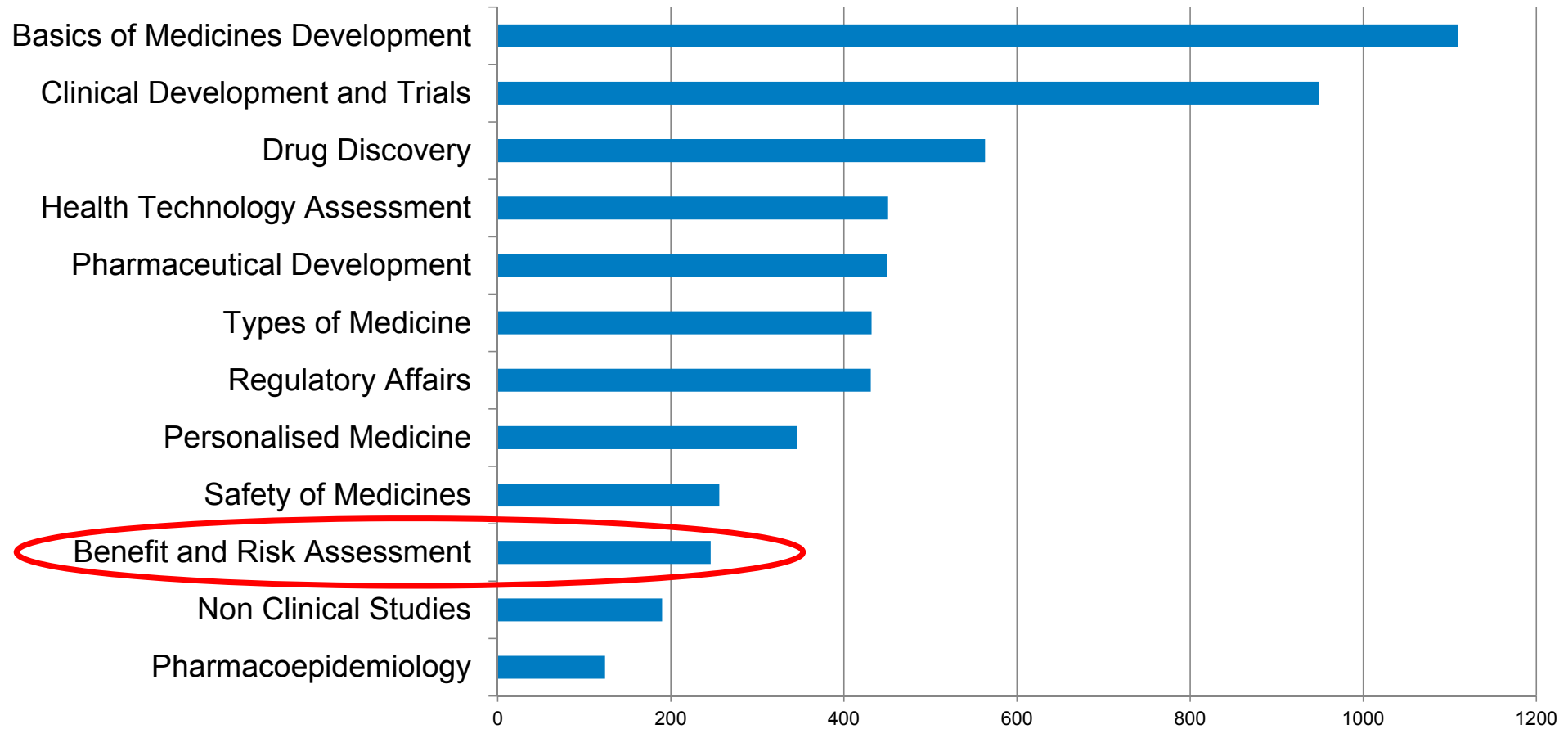


# The central role of evidence and Benefit - Risk



Different levels of tolerance for risk and uncertainty

# EUPATI website categories in order of popularity (EN)



Source: Matthias Gottwald, Bayer: EUPATI Overview for IMI 17 Nov 2016

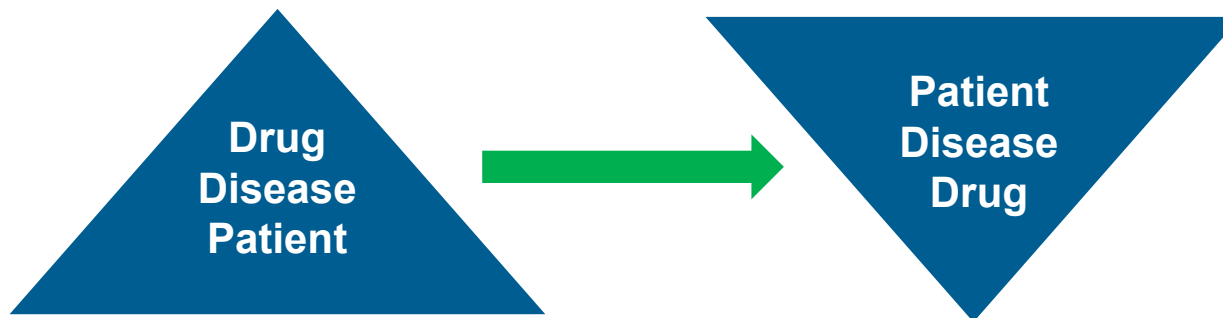
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# HOW CAN PATIENTS AND ADVOCATES DRIVE CHANGE?

# The patient role is emerging: Patient → Peer / Customer

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- Better access to health care information
- Patient network
- Increased financial involvement
- Expectation of participation



# Meaningful patient involvement

## An end to end approach

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Prevention

Screening

Diagnosis

Education

Treatment  
Mgmt

Complications

Maintenance

# EPF – EUPATI – a major initiative to empower patients through knowledge

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## EUPATI Certificate Training Programme

**Patient Ambassadors** in committees, HTA agencies, industry, regulatory bodies, academia etc  
**Patient Journalists** raising awareness  
**Patient Trainers** for patient communities and networks

**100**  
**patient advocates**



## EUPATI Educational Toolbox

Educational tools (print, slide shows, eLearning, webinars, videos) for patient advocates ( multilingual)

**12.000**  
**patient advocates**



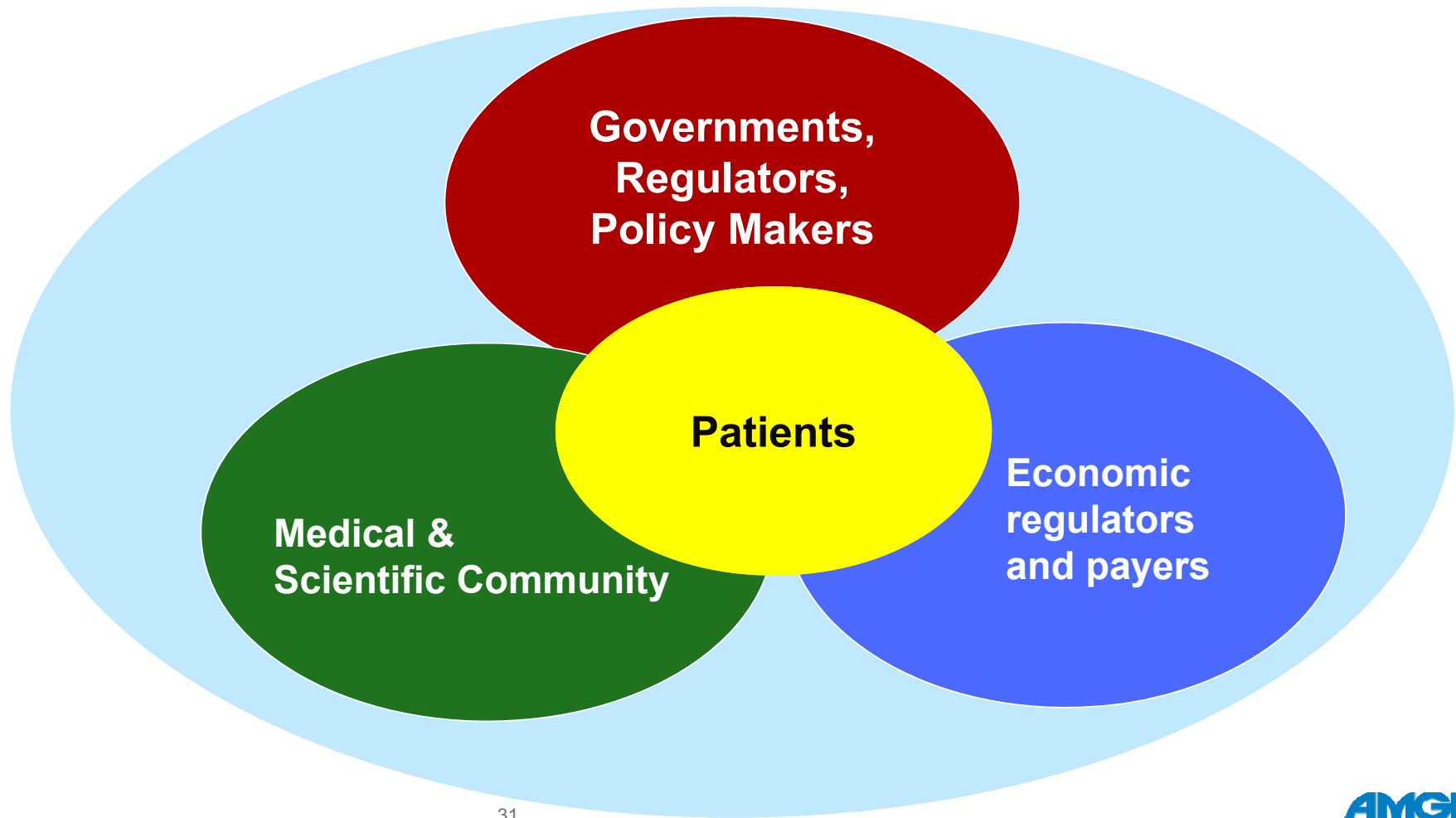
## EUPATI Internet Library

Patients & lay public at large, e.g. on specific aspects of the development process of medicines for patients with low (health) literacy (multilingual)

**100.000**  
**individuals**

# A call for patient-centric multi-stakeholder models

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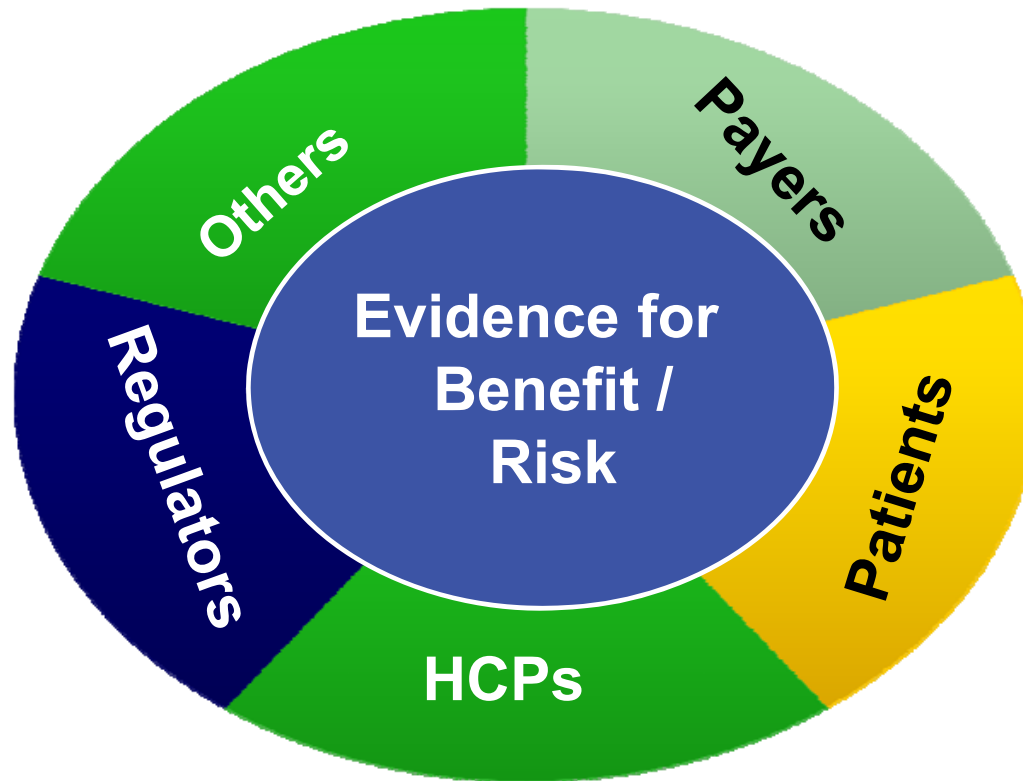
**Changing the system is tough!**

**No single stakeholder can do it alone!**



# The central role of evidence and Benefit - Risk

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Different levels of tolerance for risk and uncertainty

# A joint framework for patient engagement is needed across all stakeholders

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*Special Populations: Review*

**DIA** DEVELOP  
INNOVATE  
ADVANCE

## **Partnering With Patients in the Development and Lifecycle of Medicines: A Call for Action**

Therapeutic Innovation  
& Regulatory Science  
2015, Vol. 49(6) 929-939  
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[sagepub.com/journalsPermissions.nav](http://sagepub.com/journalsPermissions.nav)  
DOI: 10.1177/2168479015580384  
[tirs.sagepub.com](http://tirs.sagepub.com)

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## Patient focussed medicines development

Non profit, serious patient involvement

[www.pfmd.org](http://www.pfmd.org)



**IMAGINE IF...**

1 million patients were involved in  
co-designing research and the  
development  
of medicines globally

# Serving our patients is a team approach

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Thank you!

