



The European House  
Ambrosetti



Un'iniziativa sostenuta da Amgen, Celgene,  
Daiichi Sankyo, MSD, Pfizer e Sanofi

Salute, coesione e crescita del Sistema Paese

---

13 Novembre 2018 - Roma

# PRESENTAZIONE DI THOMAS ALLVIN

Executive Director Strategy and Healthcare Systems, EFPIA

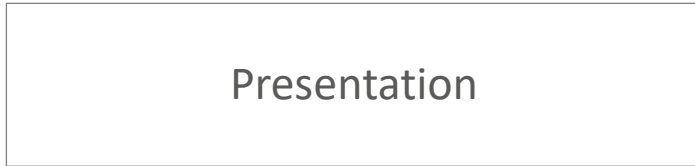


# Pharmaceutical innovation and healthcare systems

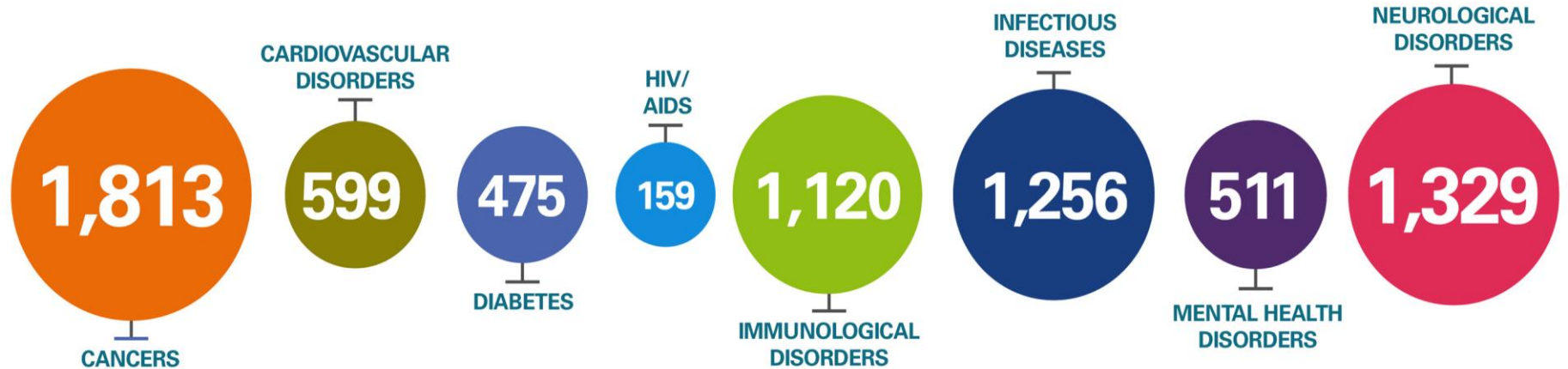
Author: Thomas Allvin

Date: November 2018

Version: Final



With over 7000 medicines in development, the exciting new wave of medical innovation will play a key role in addressing the challenges faced by patients and healthcare systems



Source: Health Advances analysis; Adis R&D Insight Database. March 2015, compiled by PhRMA

# Over the next five years many more promising medicines are expected to become available (1/2)



**CAR-T therapies** – are T-cells that have been genetically modified to allow the T-cell to recognize and destroy tumor cells



**Combination therapies** – increasing quality and quantity of life by combining targeted cancer treatments to increase their effectiveness



**Gene therapy** – helping to replace defective or missing genes in cells through the introduction of DNA for the treatment of genetic diseases



**Cell therapy** – insertion of living cells into patients to replace or repair damaged tissue, in order to facilitate improved organ or tissue functionality



**Antibacterial treatments** – neutralize highly pathogenic bacterial surface proteins or secreted toxins and activate the immune system to directly kill the bacteria



# Over the next five years many more promising medicines are expected to become available (2/2)



**NASH treatments** – reducing liver inflammation and fibrosis, to lower severity of liver damage and in some cases even reverse disease pathology



**Microbiome therapies** – readdressing the gut microbiome balance, to improve the regulation of body systems or reduce the risk of recurrence of severe gut infections



**Migraine treatments** – significantly lowering the number of days patients suffer from migraine, to improve QoL and increase autonomy



**Alzheimer's treatments** – seek to breakdown or inhibit the formation of protein plaques helping to delay the onset and progression of Alzheimer's disease



# Cell therapy can replace a lifetime of continuous insulin therapy for patient's with type 1 diabetes



## Scientific breakthrough

**Living cells are injected** into the patient to treat the causes of their diseases. This may be used to treat common conditions such as Type 1 diabetes



## Impact on patients

This will help restore **normal function of the pancreas**, reducing the patient's disease burden and the risk of serious health consequences



## Impact on health system

Cell therapy will help **control blood sugar without the need for constant insulin therapy** and help delay the onset of serious long-term health conditions



## Impact on societies

Economic productivity could increase due to reduced absenteeism and early retirement. **Welfare expenditure could also decline** if patients no longer require social care



*Approximately  
4 million people  
in Europe live with  
Type 1 diabetes*



# PPAR/FXR agonists will be the first NASH treatments available, and could significantly lower need for liver transplants



## Scientific breakthrough

Both PPAR $\alpha$ / $\beta$ / $\delta$  and FXR agonists will be the **first ever therapies** for NASH and are expected to **reduce liver fat build up**, inflammation and fibrosis



## Impact on patients

These new products promise to reverse disease progression resulting in a **significant improvement** in patient prognosis and **reducing** the need to undergo **liver transplantation**



## Impact on health system

The ability to reverse disease progression would see **fewer patients requiring costly liver transplants** and treatment associated with **liver failure complications**



## Impact on societies

The significant reduction in **debilitating complications** and time spent receiving care will reduce absenteeism, improving **economic productivity**

NASH - Non-Alcoholic Steatohepatitis



*NASH prevalence is ~15% of the general population*



*The number of late stage NASH patients could be reduced by more than 120,000 in Europe per year due to entry of PPAR and FXR agonists*



# Microbiome therapies (MBTs) will lower *C. diff* recurrence rate, improving patient outcomes and lowering costs



## Scientific breakthrough

MBT aims to establish a **healthy gut microbiome**, restoring gut function and significantly reducing risk of *C. difficile* infection recurrence



## Impact on patients

Reducing risk of life threatening *C. difficile* infection recurrence will **lower the risk of death**, as well improving long-term **quality of life** and **reducing the need for invasive** faecal transplants




## Impact on health system

MBT will **reduce the long-term costs of infection care**, through reducing patient readmissions and need to treat infection complications



## Impact on societies

Use of MBTs in combination with antibiotics will **lower the risk of antimicrobial resistance**, helping to prevent a return to the pre-antibiotic era



*There are over 250,000 *C. difficile* infections annually with a mortality rate of ~20%*



*Infection recurrence occurs in about 20% of cases, which is associated with increased severity and pre-disposition for further recurrence*





# CGRP inhibitors will provide a new treatment hope for patients, improving QoL and work productivity



## Scientific breakthrough

CGRP inhibitors work to block a key signalling process known to be involved in migraine mediation, to **attenuate or prevent** episodic or chronic **migraine**



## Impact on patients

These new therapies will offer a **new hope** to patients for whom existing medications do not work, enabling them to **regain function** and **improve their quality of life**



## Impact on health system

Higher efficacy therapies can **lower the number of primary care appointments** needed for existing patients, as well as potentially lowering the burden of **medication overuse headache**



## Impact on societies

Productivity will increase, as new therapies help to lower the number of days lost to work and school due to **presenteeism** and **absenteeism**



*Patients currently suffer a total of **3.9bn migraine days per year***



*New CGRP inhibitors could lower the total number of migraine days suffered by **~1.4bn days**, a reduction of 37%*



# The way forward – how can healthcare systems prepare?

To provide rapid, effective, fair, equitable and sustainable patient access to new medicines, healthcare systems can prepare in five areas:



Adapting regulatory pathways



Developing new ways of valuing and rewarding innovation



Creating new financing models



Evolving the way services are delivered to reflect new approaches to treatment



Enabling real world data to be captured and used to enable innovative care

# Valuing and rewarding innovation

*Increased dialogue between industry, HTA bodies and regulators*, through joint scientific advice, would ensure the right data is produced at each key decision point in the life-cycle of a medicine

*Adopting flexible P&R processes* could allow for provisional price levels and access to be provided in case of uncertainties at launch, by using methods such as predictive modelling; assessments can be updated post-launch and/or evolve over time based on RWE

---

*Innovative pricing models* would allow prices to be refined over time depending on benefit demonstrated through RWE

- *Introduction of pricing by indication* could allow for a price that better reflects the value a therapy delivers in clinical practice across specific patient populations
- *Increased use of pricing by performance* could better reward innovation by linking reimbursement rates to clinical / pharmoeconomic performance

## CASE STUDY: Combination Regimens in Oncology

- Combination regimens will be launching with limited data due to small population sizes and urgent need
- Patients are likely to benefit highly from combination regimens, although their true value will only become known after RWE is collected over time
- Prices will likely reflect the high benefit of these products, so innovative pricing and/or payment models will have to be employed

# Adapting financing models



**Integrated budgets** allow wider societal and healthcare benefits to be considered and provide sufficient resources to allow equal access without delay despite high investment



**Annuity based agreements** allow reimbursement over the duration of therapeutic benefit and ensure healthcare systems only pay for the benefit they receive



**Financial institutions** can provide insurance to cover outcome uncertainties for manufacturers and payers as well as upfront reimbursement for manufacturers

## CASE STUDY: Gene Therapy

- Gene therapy will be financed through siloed budgets in many EU markets. For example, in some countries patients can switch insurer every year so funding gene therapy can disadvantage insurers, despite benefits being felt across time and companies
- Upfront reimbursement for gene therapy is not often feasible as limited evidence of lasting clinical or economic benefit is available at launch leading to uncertainty around value

# Developing novel, integrated care delivery pathways

Not all aspects of innovations fit the traditional pharmaceutical delivery pathway, therefore the **development of new and upgrading existing infrastructure and pathways** is necessary



**Integrated care and manufacturing facilities** could see cell and gene therapies produced and delivered at centres of excellence in order to reduce turn around time and enable collection of reliable patient outcomes data



**Early-diagnosis centres** with diagnosis labs and are needed to fully realise the value of certain therapies, such as disease modifying therapies in Alzheimer's or diagnostic tools for cancer before patients and healthcare systems are burdened by the long term consequences



**Restructured care pathways** will be vital to accommodate the influx of new patients created by new innovations that provide a treatment where previously none were available or effective, such as NASH treatments and CGRP inhibitors for migraine

## CASE STUDY: CAR-Ts

- First-generation CAR-Ts are allogenic, where blood cells from a patient are harvested, transported to the lab, processed and sent back to the patient
- Significant development in care delivery infrastructure is required to provide to enable fast treatment, otherwise patients with aggressive cancers may not be able to access treatment in time to realize the benefits and long term value expected
- Manufacturing of CAR-Ts may be needed at or close to the point of delivery to ensure their value is realised

# Development of real world data capture systems and updating data policy



*Proactively adopting eHealth / mHealth initiatives* would provide healthcare systems access to robust data capture systems needed to generate data on patient outcomes



*Continued revision and standardisation of national / international policies for data collection and re-use* while fully respecting legislation on data protection in order to allow for richer real world data sets to be captured and shared for analysis



*Collaboration between healthcare system stakeholders and manufacturers* is critical to ensure the relevance and development of RWE systems

## **CASE STUDY: Alzheimer's Disease Modifying Therapy**

- Early detection of Alzheimer's is expected to be crucial to the success of disease modifying therapy
- Access to rich high-volume data sets could allow predictive analytic techniques to identify key risk-variables and mHealth platforms could allow them to be monitored in real-time based on a user's digital behaviour over time as the disease progresses

# Horizon scanning will be vital for payers and providers to ensure awareness is high and that new innovations are planned for

- \* Robust horizon scanning at country level is needed to help healthcare systems plan and prepare for new innovation
- \* The industry is ready to help health authorities with horizon scanning to understand what is coming through the industry pipeline
- \* Horizon scanning should not be an isolated exercise – but inform policymaking, healthcare service management and budget planning
- \* Given the wide range of issues involved, all stakeholders should be involved in implications and planning discussions

## CASE STUDY: CGRP inhibitors for migraine

- CGRP inhibitors are anticipated to significantly increase the number of treated patients, through offering a new mechanism of action as well as improved efficacy over existing options
- These therapies will also be initiated in secondary care setting, whereas existing treatments are generally initiated in primary care
- Therefore, ahead of their launch, healthcare systems have started to plan how care pathways will be structured (e.g. in UK, plans are already in place to ensure care is delivered through specialised hubs)

# Stakeholder collaboration across healthcare and industry will be required to realise benefits of innovation

