Zorginstituut Nederland

Medicinal products in 2030

A feverish futuristic dream

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Two comments on the title and disclaimer

This is looking back from the future 2030, therefore no typo

In the 2010s, only 40% of new registrations were orphan indications
But by now it is 80 %

This presentation is a scetch of a possible future. It does not represent the views of the Health Care Institute, nor its policy agenda. The scenario's & ideas expressed are not even those of the speaker, and only serve to stimulate discussion

Political and economic unrest has changed attitude to willingness to pay

EU dominated by foreign power that exerts de facto political power

US have practically imploded and concentrate on protecting their own soil

EU economy has weakened considerably, medical budgets suffer in consequence

Although the EU has proved governmentally resilient, it is full of political unrest, with suicide attcks taking place daily, loss of human life has become commonplace

This has changed outlook on value of life. Hardly anyone still remembers that it was common in the 2010s to reimburse some drugs at the rate of half a million per patient per year.

Explosion achieved in new therapeutic approaches

Techniques to produce active material no longer take up much development time

ATMPs and drugs are produced by modular systems that produce limited amounts on demand

Protracted clinical trials have become unneccessary

There is a new rate limiting step for full acceptance of a drug by payers: proof of cost effective health gains

Personalization of treatment

Has been progressing rapidly since Google started to collect large amounts of patient data from hospitals, iphones, ipads and wearables (mobile health)

Many people have lost all sense of privacy in this respect, or do not realize to a sufficient extent that it is happening

Gone very fast since electronic purchasing data from Tesco were coupled to healthcare databases back in 2017

Big data approach to the frequent health problems

Watson-like approaches have unraveled many causes of big diseases like diabetes and high blood pressure and have triggered both behavioral interventions and personalised application of generally used medications

That is more difficult for rare diseases

Obsolete example dating back to 2017: the chip inplanted in aripiprazole that sends a signal to your iphone and your doctor once the pill is activated by the acid environment in the stomach

Time to registration vs time to reimbursement

Time to registration becomes shorter because swifter development processes

Time to reimbursement becomes longer because preparation for data registration and negotiation process costs a lot of time

Only limited net shortening of the time to patients

Reimbursement condition in rare diseases

There must be a EU wide disease database enabling regular data uploading by local expert treatment centres in MS

The database can be queried any time by payers, physicians and other stakeholders

Database managers produce a public annual report containing at least chapters on outcomes and Quality of Life.

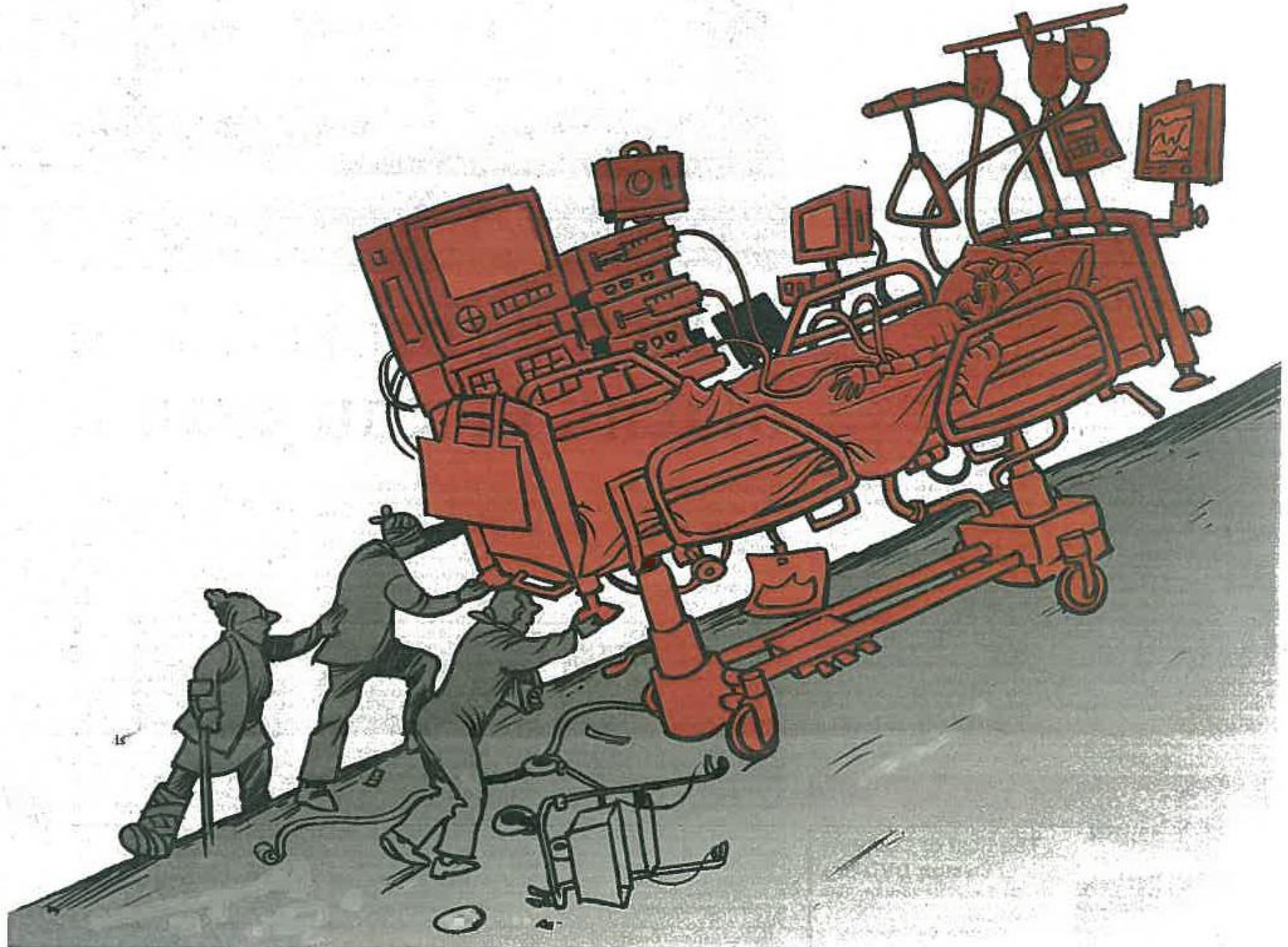
The speculative development model of the 2020s

You are a big pharma company
You spot a promising small company that has a promising new product
This product is 1 to 2 years from registration
A product from the same class is turning over 6 million € a year

You make a bid of € 7 billion

It is accepted

What do you think will drive the pricing of this product?



Negotiations

Negotiations will take place EU wide (multistate procurement) unless MS exercise opt-out right

If MS participate in EU negotiations, they must adopt the EU price.

However, HTAs can advise to restrict access on a national level

Poorer countries can apply for support funding from a EU fund

EU fund for poorer countries

There used to be a EU community for coal and steel called EGKS

But now health has become one of the most important economic resources in a EU with a diminishing population

Therefore a fund is necessary to make sure that also the poorer countries can access new healthcare products like drugs and diagnostics

However, MS can only apply for funding if they participate in and recognize EU wide HTA assessments

It was the best carrot the Commission could find

Since public healthcare is a public good

….it is incompatible with intransparency and secrecy, leading to a decline in managed entry agreements

Value is not obscured by high prices but by excessive profits, leading to the first time application of a provision in the orphan drug regulation that an orphan status can be revoked.

Companies declining to justify prices got along fine until 2020

But after that, more rejections took place based on a stricter adherence to ICER reference values

Also the concept of displacement (Claxton) became generally accepted

Moreover, the stacking of copays not only for medicinal

products but also for supporting daily activities got recognized

A real shock: compulsory licensing comes to EU

In 2022, Greece was the first member state to introduce compulsory licensing, to treat all fugitives permanently residing on their soil for sickle cell aneamia.

Many member states follow suit

Fall of negotiated prices

Role of EMA

EMA primarily checks on safety and proof of principle. Efficacy is no longer strictly demanded

EMA proposes proven off label applications to the originator to register. If they refuse, the indication will be offered to other potential takers

Quid pro quo: longer exclusivity of data, or indications in return for willingness to register hardly ever accepted

Unmet medical need

EMA finally got round to defining unmet medical need

Takes into account the existence of current treatment options

No longer defined as “room for improvement”

Role of the scientific advisory board (WAR) as an assessment body

Role of the WAR has dwindled

Too many new drugs and individualized treatments come without sufficient underpinning of effectiveness

Orphanisation of innovation: many registrations conditional or under exceptional circumstances

Role partly absorbed by EUNetHTA since this procedure is now in many cases compulsory

Example from 2014

Cost of expensive drugs	: € 1,7 billion
General hospitals	: € 1,1 billion
Teaching hospitals	: € 0,56 billion
Outpatient pharma market:	: € 4,0 billion
Annual growth rate	: 5 -10 %
Politically agreed growth rate	: 1%

Equates to ~60 million

Advisory Committee to the Package ACP for societal debate (appraisal)

Works with an annual innovation agenda

Collects new assessment outcomes during the year

Ranks these according to real contributions to care and budget impact taking into account displacement effects, cost per QALY and QoL

Ranking takes place in an all-day meeting with input from stakeholders

On the week after, final ranking takes place in a public session without Outside contributions

National Healthcare Institute (ZIN)

Has enforced policy of additional arguments needed to exceed reference values for cost effectiviness.

If companies refuse to explain price setting, no waiver of e.g. € 80.000 ceiling

On the other hand, value is calculated without any regard to previously existing firewalls and includes societal gains.

Patients

Develop more rational attitude to health gains

Have become more integrated in drug development process

Have become a force to remind industry of its past philantropic attitude

Old atmosphere of George Merck: “look after the patients first and then the profits will look afer themselves” slowly returning

Insurers

Have become bankers

Look at desired future outcome measures and start with a default reimbursement level and pay additional money later if pre-agreed outcome thresholds have been reached

For this, they have to build up huge financial reserves, necessitating rise in premium

Health ministry

Fixes an annual amount (investment headroom) for new interventions and treatments

Has final responsibility to remain within financial boundaries by acting on ZIN suggestions

Works with internationally negotiated prices

But is not obliged to include every new intervention with a negotiated price into the insured package

Conclusions

Increased austerity balanced by broader valuation of societal health gains

More rapid flow of new products

More rational approach to investment in healthcare

More account taken of displacement