

Experiences of a patient organisation at the national level.

The patient as the third party:
introduction to the present situation

“no policy without patients”
Maastricht 30 October 2008





The goals of the CKP are:

- Stimulate contact between patients
- Give information to the patients
- Promotion of interests of the patients
 - On national level
 - On international level



Promotion of interests of the patients

The patient organisation must act with other organisations and the authorities for the benefit of the patients interests.

They must do so on local level, national level and international level.

“The Thalidomide case” is a good example and gives insight in the abilities of the organisation and the pitfalls.



The Thalidomide case: the history

In 1964 Israeli physician Jacob Sheskin was trying to help a critically ill patient with erythema nodosum leprosum (ENL), a painful complication of leprosy.

He searched his small hospital for anything to help his patient stop aching long enough to sleep. He found a bottle of thalidomide tablets, and remembered that it had been effective in helping mentally ill patients sleep, and also that it was banned.

Sheskin administered two tablets of thalidomide, and the patient slept for hours, and was able to get out of bed without aid upon awakening.

The result was followed by a clinical trial. Dr. Sheskin's drug of last resort revolutionized the care of leprosy and led to the closing of most leprosy hospitals.



The Thalidomide case: the history

On Friday, [Sept. 19, 1998](#) the Food and Drug Administration issued an "approvable" letter to Celgene Inc. for thalidomide as a treatment for a leprosy complication: the skin manifestations of erythema nodosum leprosum.

First clinical study in the medical literature about the usefulness of Thalidomide in multiple myeloma was in [1999](#).(New Eng. J. Med)

On [May 26, 2006](#), the U.S. Food and Drug Administration granted [accelerated](#) approval for thalidomide (THALOMID®) in combination with dexamethasone for the treatment of newly diagnosed multiple myeloma (MM) patients.

Thalidomide full registration for the EU for the treatment of previous untreated multiple myeloma patients [21-4-2008](#)



Thalidomide implementation in The Netherlands

What was the problem?

Clinical results were so good that:

- Patients wished that the drug was available to them
- Doctors were eager to prescribe

Negative aspects

- History of the drug
- No registration
- Thalidomide via Pharmion was difficult to obtain (and expensive).
- Celgene US made an unacceptable Risk Management Program

Positive aspects

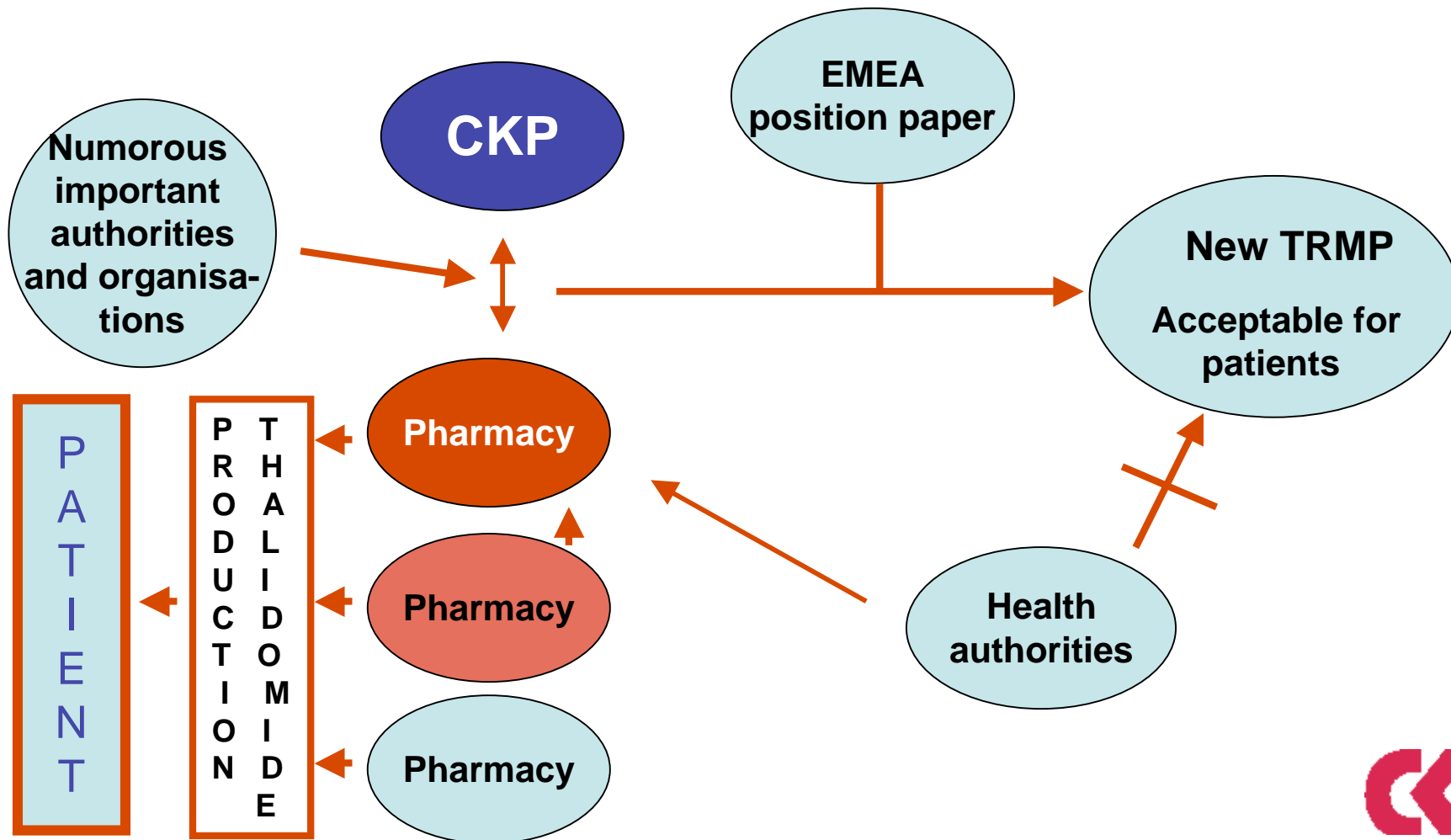
- Patients were aware of potential risks
- Dutch law made it possible for pharmacists to deliver Thalidomide (and cheap).



The Thalidomide case.

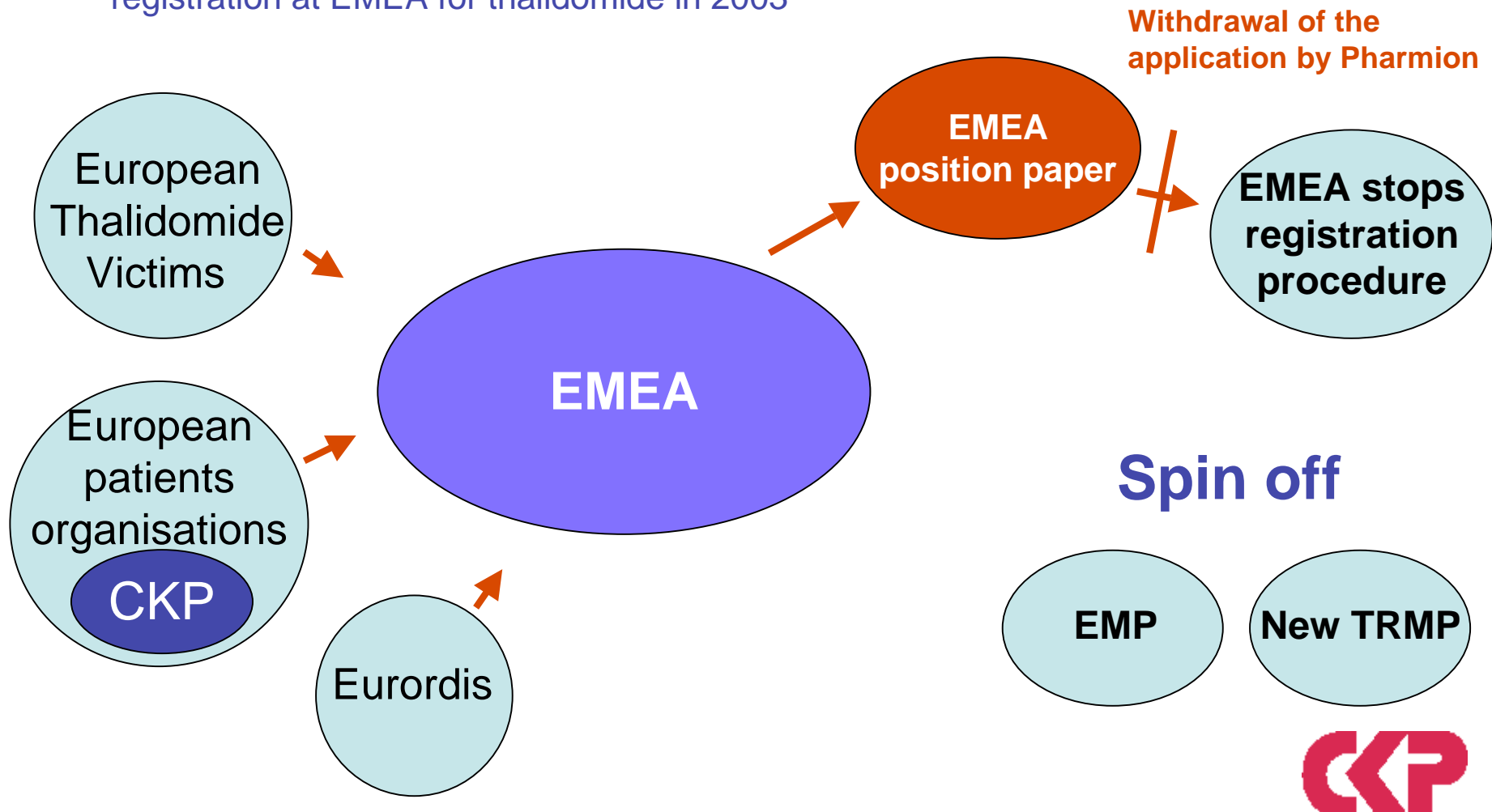
CKP actions national

Most European countries await anxiously the EU registration, in the Netherlands there is a different situation.



The Thalidomide case. CKP actions international

Most European countries await anxiously the EU registration. Application for a European registration at EMEA for thalidomide in 2003



The Thalidomide case: what did we learn?

Patients are well informed.

Patients are reasonable; they do understand registration problems.

Patients do not understand that a drug is available in country A but not in country B.

Patient's groups are able to work together with authorities.

Patient's groups are able to mobilise from there groups capable Individuals to discuss their interests.

Patients are eager to co-operate with study groups in the development of new drugs.



Patients are well informed!

Google

Search items: Multiple Myeloma, new drugs: ca 187.000 hits

Multipel myeloom, nieuwe geneesmiddelen ca 1.600 hits

Waldenstroms Macroglobulinemia
new drugs ca 11.600 hits

Ziekte van Waldenstrom,
nieuwe geneesmiddelen 542 hits

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Genomic Health makes a \$3,480 test to help determine whether breast cancer patients would benefit from chemotherapy. UnitedHealthcare, a U.S. insurance company, is trying a risk-sharing experiment that would tie test effectiveness with its price. (Peter DaSilva/for the New York Times)

Drug company offers money-back guarantee on cancer treatment in Britain

By Andrew Pollack

Published: July 13, 2007

NEW YORK: Drug companies like to say that their most expensive products are worth their breathtaking prices. Now, one company is putting its money where its mouth is by offering a money-back guarantee.

The company, Johnson & Johnson, has proposed that Britain's national health care system reimburse use of the cancer drug Velcade, but only for people who benefit from the medicine. The company would refund any money spent on patients whose tumors don't shrink sufficiently after a trial treatment, which can cost \$48,000 per patient.

The ground-breaking proposal, along with less radical pricing experiments in the United States and around the world, may signal a willingness in the pharmaceutical industry to edge toward a new pay-for-performance paradigm in which the price of a drug would be based on how well it works, and might be adjusted up or down as new evidence is released.

"I think payers will say, 'If the product works and it creates value, we will reward you for it,'"

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Start Thalidomide therapy in multiple myeloma	1999
All hematologists convinced about usefulness	2004
FDA registration	2006
EMA registration	2008

Thalidomide is not the only drug that is registered by the FDA long before the EMA registration takes place.

Azacitidine	FDA approval	2004
	EMA approval	not yet
Decitabine	FDA approval	2006
	EMA approval	not yet

When a drug is approved it takes ca 1 year before it is available for the Dutch patient.



From 1956 to 1962, approximately 10,000 children in Africa and Europe were born with severe malformities, because their mothers had taken thalidomide during pregnancy. The impact in the US was minimized when [Frances Oldham Kelsey](#) refused FDA-approval for an application to market it saying it needed more study. Only 17 children in the U.S. were born with the defects.





Regulation Policy

The FDA's Thalidomide Record

When a German scientist identified the risks of birth defects due to Thalidomide some 40 years ago, the Food and Drug Administration congratulated itself for its failure to approve sales of the drug in the U.S. But those familiar with the case say the FDA had literally done nothing. A junior official at the agency had simply dawdled over the drug manufacturer's application, thus delaying approval.

Those who understand the approval process contend that if Thalidomide proves successful in combating these other conditions, the successes won't make it onto the label for years, if ever.

Source: Peter Huber (Manhattan Institute), "FDA Caution Can Be Deadly, Too," *Wall Street Journal*, July 24, 1998.



Organizing patient's organisations in The Netherlands.

CKP: Dutch Multiple Myeloma and Waldenstroms Macroglobulinemia patient organisation.

Acts with other patients organisations for hematological malignancies.

Because there are so many patients groups the authorities would like to have one discussion partner.

Patients groups are organised within the NFK

NFK is a growing organisation thus the following problems might occur:

- Smaller groups have different needs compared to well organised groups
- Larger groups do have more influence.
- NFK tends to act independently from the patients organisations.
- As soon as more money is involved more independent minds take over the agenda.



The Thalidomide case: what are the pitfalls?

Patients want results too quick.

Patients must be well organised to be heard.

Patient's organisations lack power and money.

Patients umbrella organisations are working independently of patients advocacy groups

Pharmaceutical industries are eager to influence the process by “helping” the patient's organisations.



What would the CKP like to achieve in the near future

National

The CKP needs everybody's help to come to:

- Echelonment of hospitals taking care of myeloma patients.
- New therapies available as soon as EMEA has given approval.

International

The CKP would like to cooperate with other groups to come to

- Harmonization of laws and rules in the EU
- Cooperation between FDA and EMEA
- Rules for the influence of the pharmaceutical industries
- Involvement in the research to new therapeutic modalities



Thank you for your attention!

Special thanks to: Arno Baumann
Lia van Ginneken



De NFK is de koepelorganisatie van 25 [kankerpatiëntenorganisaties](#) in Nederland. Deze organisaties bieden lotgenotencontact en informatie rond bepaalde vormen van kanker of een bepaalde problematiek. Samen met patiëntenorganisaties behartigt NFK de belangen van (ex-)kankerpatiënten in Nederland. De NFK werkt samen met en wordt financieel gesteund door [KWF Kankerbestrijding](#).

Stichting Fonds PGO verstrekt subsidies aan landelijk werkzame patiëntenorganisaties, gehandicaptenorganisaties en ouderenbonden in Nederland. Het Fonds PGO wordt hiertoe in de gelegenheid gesteld dankzij een jaarlijkse bijdrage van het Ministerie van VWS.

Directeur Ferdinand Clevers van het Fonds PGO mist het patiëntenperspectief in de verkiezingsprogramma's van de verschillende partijen.

“Voor de patiënt is de zorg nog altijd een black box”, aldus Clevers.

“Zolang de patiënt niet kan beschikken over transparante informatie kan erg een sprake zijn van echte marktwerking. Dat is een fundamenteel manco in het nieuwe stelsel.”

