EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Unproven cell-based therapies: a risk to public health and to the development of advanced therapies

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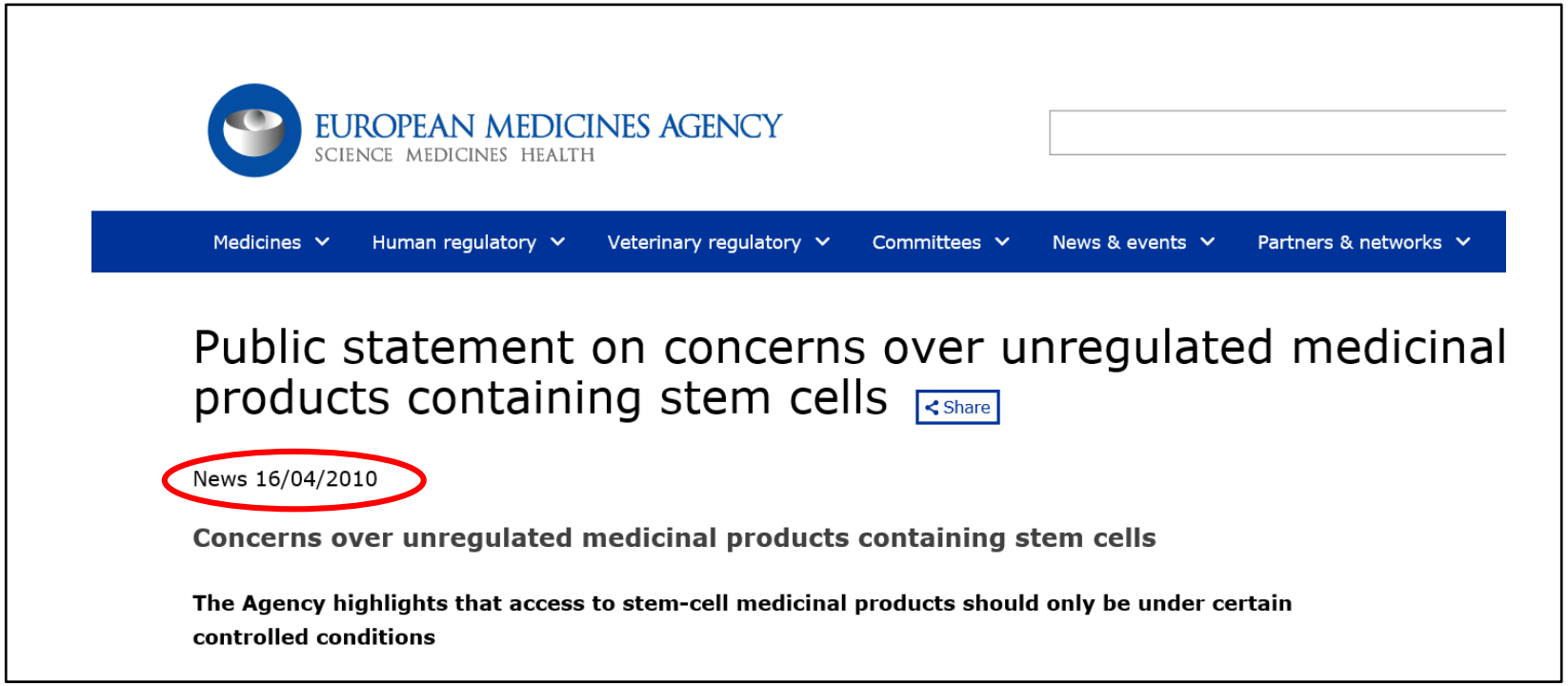
Cell-based therapies

- Cell-based medicinal products have the potential to address some of today's unmet medical needs
- These products are currently regulated under the medicinal products framework in the EU
- The Regulation on Advanced Therapy Medicinal Products (ATMPs) has supported the successful development of innovative medicinal products
- EMA strategy core recommendations: "ATMP development"
- For the field to deliver on expectations, it is **essential** to adhere to the principles that have permitted the advancement of medicine

Unregulated medicinal products



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Public statement on concerns over unregulated medicinal products containing stem cells

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News 16/04/2010

Concerns over unregulated medicinal products containing stem cells

The Agency highlights that access to stem-cell medicinal products should only be under certain controlled conditions

Challlenges for cell-based therapies

- Patients are still being offered **unproven and/or unauthorised cell-based therapies** which are presented as curative for a broad range of conditions such as autism, cerebral palsy, muscular dystrophy and vision loss
- While this has been traditionally associated with so-called '**stem cell tourism**' in countries lacking robust regulatory frameworks, the EU has not been spared
- Role of the Hospital Exemption Scheme for initial product development
- Significant impact of any change in cell therapy regulations at a European level

X-cell centre

British lab working with controversial stem cell doctor

16 April 2012

By Ana Pallesen

Appeared in BioNews 652

A commercial London laboratory is working with a clinic in Lebanon that uses medical techniques that are not verified by the medical community, and fall outside most countries' legal parameters.

The Cells4health clinic is run by Dr Cornelis Kleinbloesem, former owner of the XCell-Center in Germany, which was shut down in 2011 after a child died following a stem cell injection into his brain, according to the Telegraph. A legal loophole is now allowing Dr Kleinbloesem to operate his clinic in Beirut.

Dr Kleinbloesem's clinic takes cells from the patient's bone marrow and sends them to the UK Precious Cells laboratory for processing. After this, the stem cells are sent back to Lebanon and injected into the patient to regenerate damaged tissue. Precious Cells is licensed by the Human Tissue Authority to extract and bank stem cells, but could not legally use them to treat patients in the UK.

Stem cell clinics - US

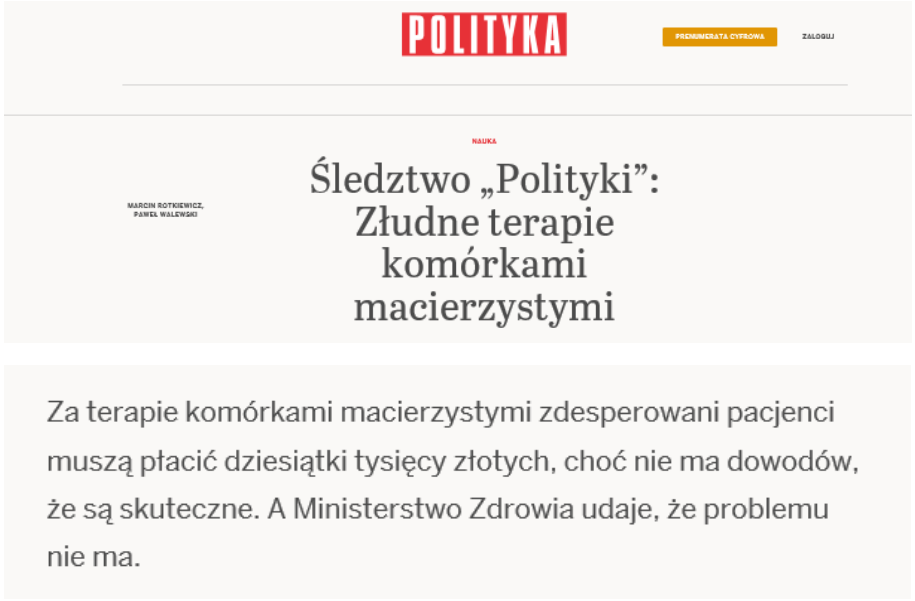
Vision Loss after Intravitreal Injection of Autologous "Stem Cells" for AMD

Kuriyan, Ajay E; Albini, Thomas A; Townsend, Justin H; Rodriguez Marianeli; Pandya, Hemang K; et al.

The New England Journal of Medicine; Boston Vol. 376, Iss. 11, (Mar 16, 2017): 1047-1053.
DOI:10.1056/NEJMoa1609583

The outcome of poor visual acuity in these three patients arouses concern about the performance of procedures at stem-cell clinics that charge patients for their services and that lack clinical or preclinical data to support their practices. Many stem-cell clinics have claimed that autologous stem-cell injections do not fall under the auspices of FDA regulations.[7–10] Our case reports establish the possibility of devastating outcomes from such procedures. Other case reports have shown a lack of efficacy in cases of medical tourism in which nothing was injected into the patient's eye.[8] The need for oversight of such clinics and for the education of patients by physicians and regulatory bodies is paramount to protecting patients while advancing proper research and innovation.[22–24]

Stem cell clinics – Europe



POLITYKA

PRENUMERATA CYFROWA ZALOGUJ

NAUKA

Śledztwo „Polityki”: Złudne terapie komórkami macierzystymi

MARCIN ROTKIEWICZ, PAWEŁ WALEWSKI

Za terapie komórkami macierzystymi zdesperowani pacjenci muszą płacić dziesiątki tysięcy złotych, choć nie ma dowodów, że są skuteczne. A Ministerstwo Zdrowia udaje, że problemu nie ma.

"Polityka" investigation: Deceptive stem cell therapies

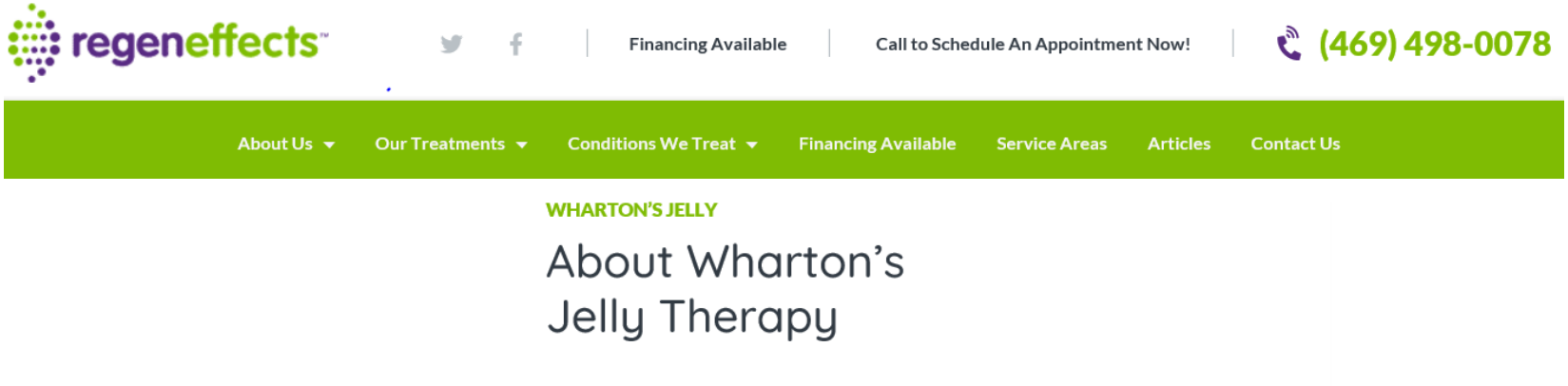
Desperate patients must pay tens of thousands of zlotys for stem cell therapies, although there is no evidence that they are effective. And the Ministry of Health pretends that there is no problem.

Stem cell product classification

The CAT has been confronted with an increasing number of applications for scientific recommendation on classification for cell-based products to treat a variety of conditions but for which neither a **plausible mechanism of action** nor any other **scientific evidence on the safety and efficacy** has been presented.

CAT minutes Oct 2019

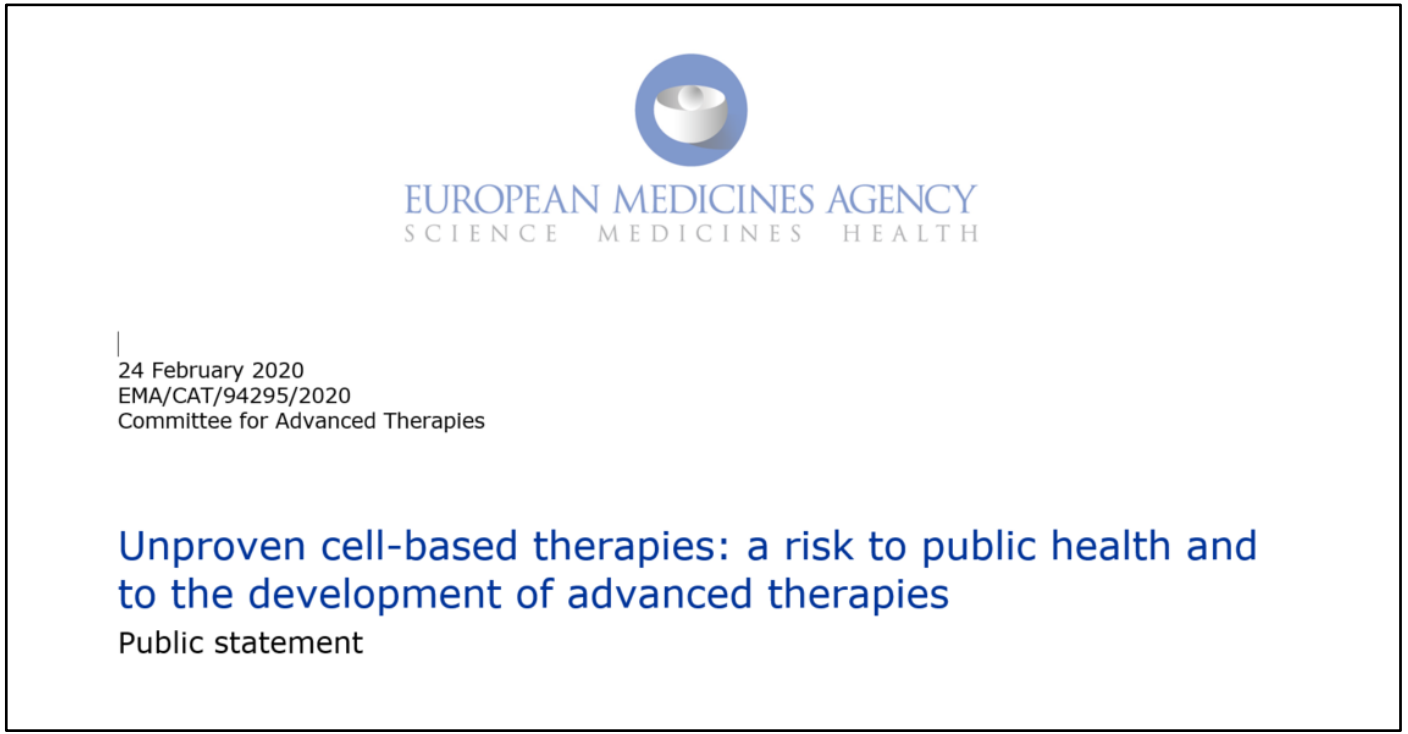
Stem cell products



Future therapies

- Rapidly evolving technology in the fields of gene and cell therapy brings exciting new opportunities for treating and ultimately curing a range of diseases
- Circumvention of the marketing and clinical trial authorisation procedures makes it difficult to understand and document the safety and efficacy profile of these treatments
- This can discredit the entire field and put properly conducted medical research at risk, thereby depriving future patients of the possibility of access to truly curative products.
- In order to protect public health in the EU and to support the development of these promising treatments, the field of cell-based therapies needs rigorous scientific control and evidence-based regulatory oversight.

CAT Public statement 2020



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Public statement