



Primjena biotehnologije u genomskoj medicini

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Biotehnologija

biotechnology
new horizons in medicine

Biotechnology is the use of biological organisms or enzymes in the synthesis, breakdown or transformation of materials in the service of people. People have used biotechnology for thousands of years to make bread, cheeses, wine and beer, but in the last century, since the discovery of antibiotics, things have moved on rapidly. In the 21st century, most of the medical developments with the greatest potential to improve human health involve biotechnology.

Making medicines
Biotechnology has given us genetically engineered organisms ranging from bacteria to cows and sheep, producing life-saving medicines including vaccines and blood-clotting factors. Genetically modified bacteria can make the lung surfactant needed for premature babies to survive and the insulin needed by thousands of people with diabetes. Monoclonal antibodies can be made to carry medicines to targeted cells.

Personalised medicines
Our ability to read and analyse the human genome is developing all the time. Results from the Human Genome Project, the 1000 Genomes Project and the 100,000 Genomes Project are being used to design specifically targeted therapeutic medicines. Our growing understanding of the human genome is leading the way to the development of personalised medicine. Future biotechnological developments may lead to individually designed medicines. These will interact with our personal genetic combinations enabling doctors to treat our diseases more effectively and to minimise any side effects.

Reading the genes
The speed and accuracy with which we can analyse genomes from pathogens to people has increased over the last 25 years by orders of magnitude. Analysing the first human genome took years - now it takes days and is decreasing continuously. Developments in both computer technology and biology give us an ever-increasing ability to collect and analyse big data fast. Knowledge of the human genome has resulted in the development of gene probes to test for genetic diseases. In future they may be used to diagnose genetic tendencies towards problems such as cancer or heart disease. We can also use genome sequencing to identify pathogens, allowing us to treat them effectively and track infection routes.

New parts for old
Some of the latest developments in biotechnology involve stem cells, derived from embryos or adults. They have the potential to grow and develop into new tissues or organs which can be used to replace others which are worn out or diseased. Trials using stem cells are taking place to try and restore lost sight, and to repair damaged hearts and damaged tendons. The technology is at a very early stage but has massive potential for the future.

Testing, testing
Medicine benefits from many sensitive tests which indicate the presence or absence of substances like sugar or hormones in body fluids. Biotechnological advances in the use of immobilised enzymes and monoclonal antibodies mean these tests have become very rapid and accurate in recent years. A pregnancy test used to take weeks - it now takes minutes!

Mending the genes
Gene therapy is one area of medical biotechnology which is still in the very early stages of development. The hope is that gene technology will help scientists develop ways to correct the mistakes in the DNA code which lead to genetic diseases. Research and trials are under way to treat conditions ranging from cystic fibrosis and severe combined immunodeficiency (SCID) to rare forms of inherited blindness.

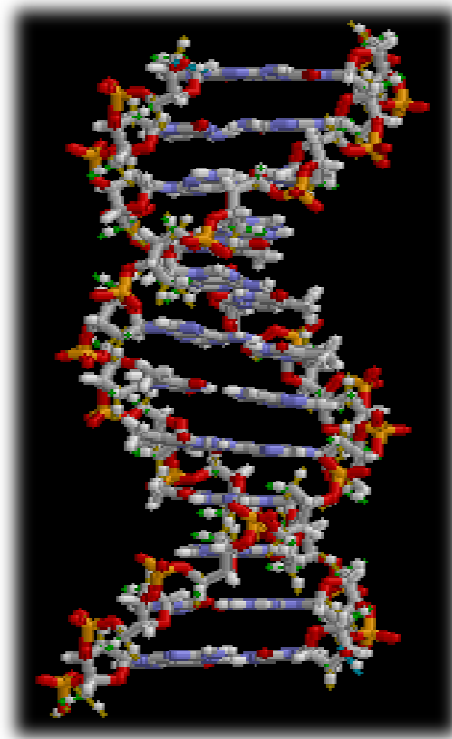
abpi
Bringing medicines to life

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- Antibiotici
- Primjenjena genomika
- Genska terapija

Medicinska biotehnologija



Genetičko inženjerstvo 1982.

Hormoni, faktori koagulacije, cijepliva,
matične stanice, tkiva, organi



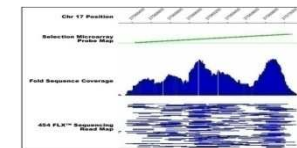
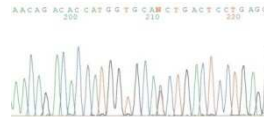
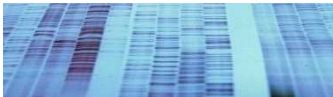
Restriksijski enzimi
1970.

Genetički *fingerprinting*
1984.

Lančana reakcija polimerazom – PCR

Kary B Mullis, 1983.

Sekvenciranje DNA

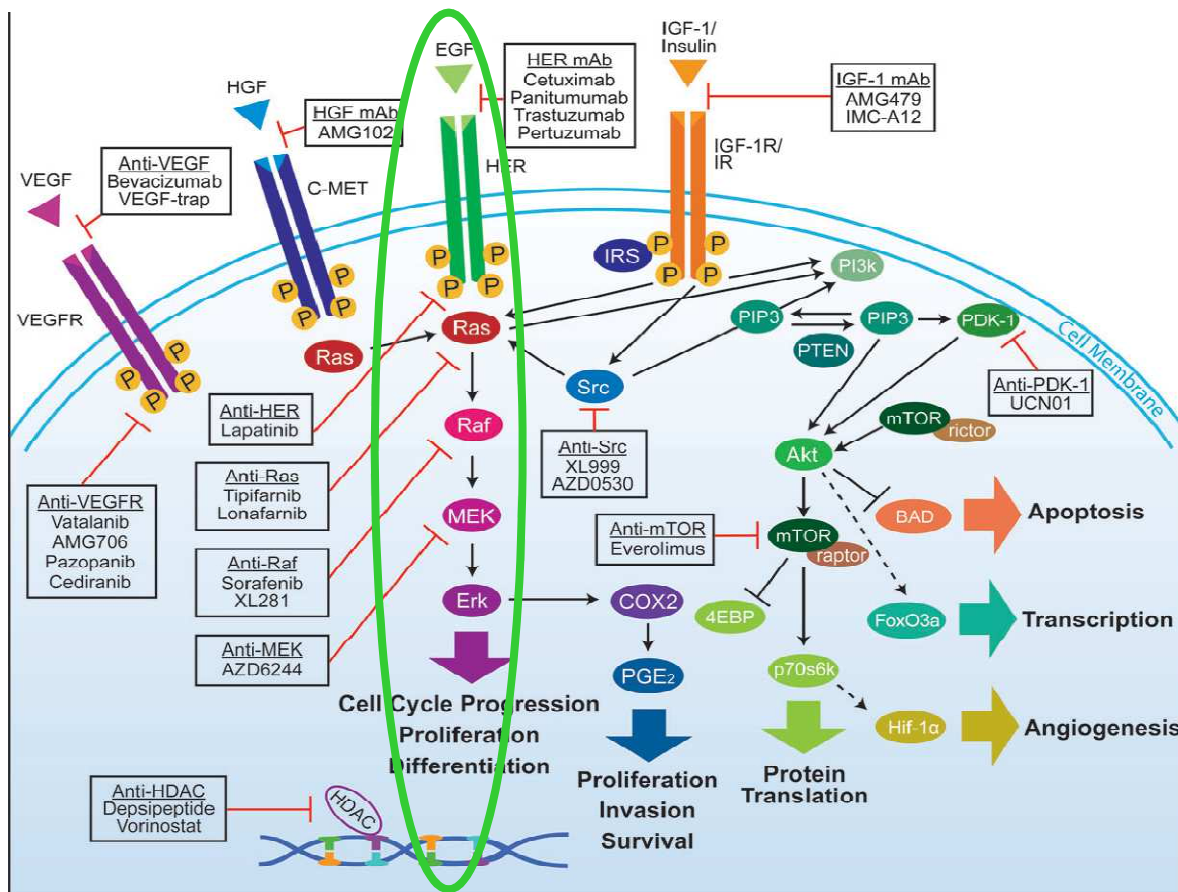


Sekvenciranje humanog genoma

EU – projekt

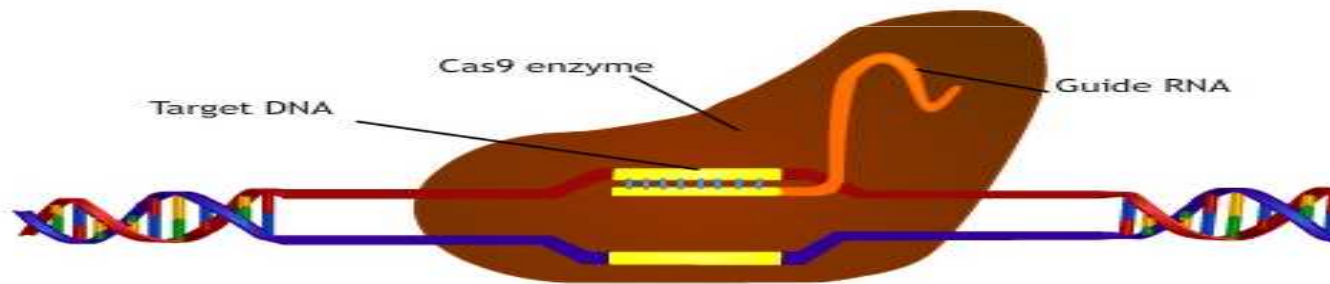
"Towards access to 1 million Genomes in the EU by 2022".

Biološki lijekovi – monoklonska antitijela



Jones S i sur. PNAS 2008;105:4283-8.

Editiranje gena – CRISPR-Cas9 2016.



Biochip



PRIMIENJENA GENOMIKA

– precizna medicina

urednici: Jadranka Sertić, Stjepan Gamulin, Filip Sedlić



MEDICINSKA
NAKLADA

Personalizirana medicina

➤ **Spinalna mišična atrofija** – *protusmjerni oligonukleotidi*

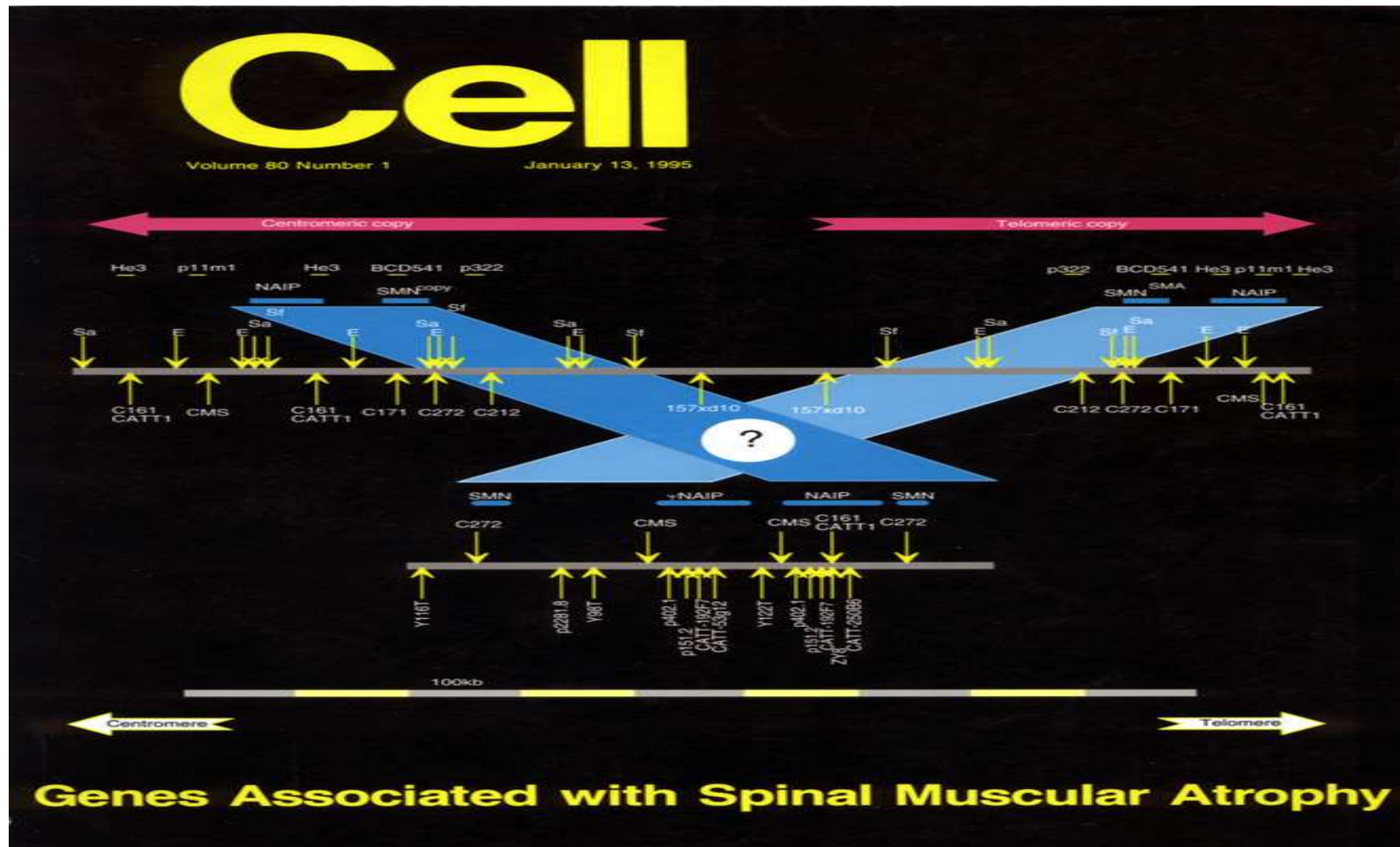
➤ **Cistična fibroza** – *terapija prema mutacijama*

➤ **Multipla endokrina neoplazija tipa 2** – *tireoidektomija*

➤ **Imunodeficijencija SCID** – *transplantacija koštane srži*

➤ **Monogeniski dijabetes (precizni dijabetes)** – *inzulin/sulfonilurea*

Spinalna mišićna atrofija - *spinraza*



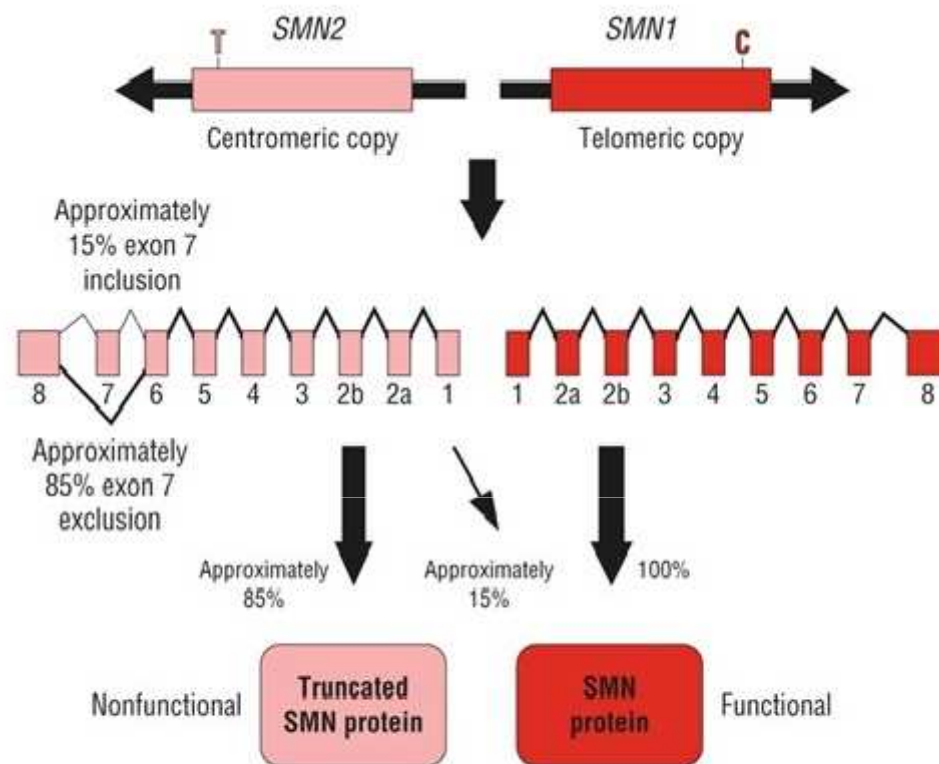
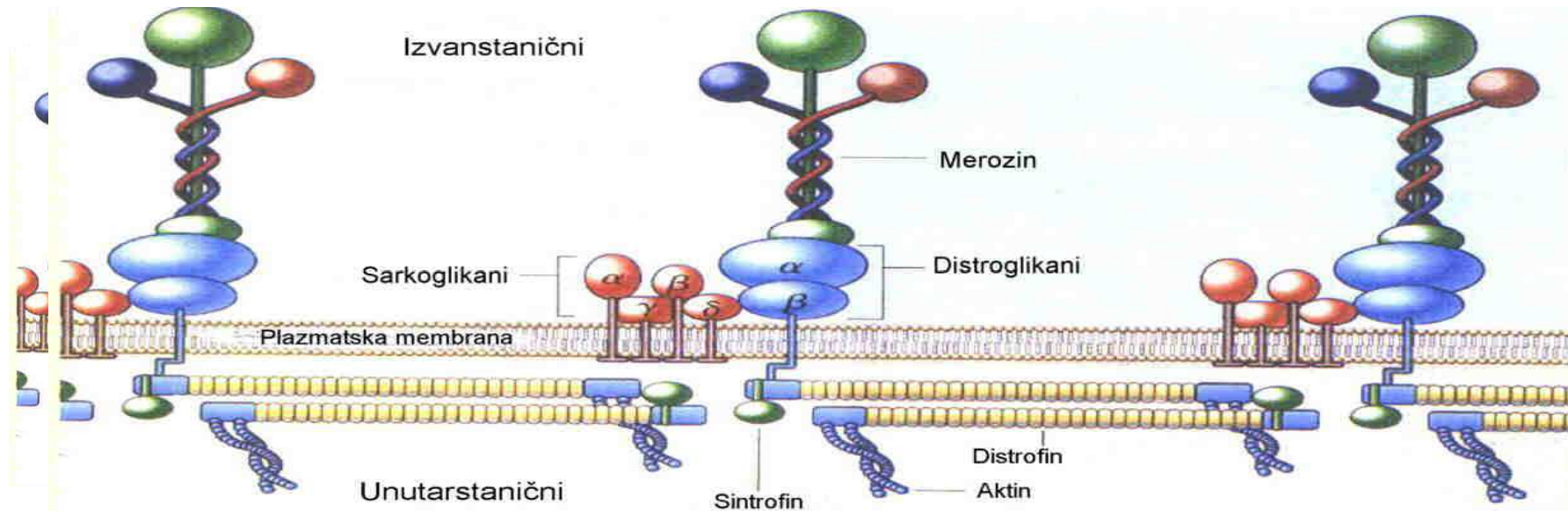


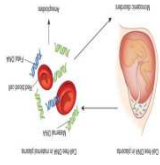
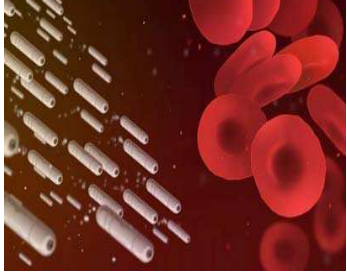
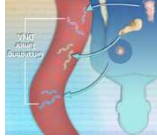
Image credit: Stephen J. Kolb, MD, PhD; John T. Kissel, MD; Wikimedia CC

DMD distrofin - 30 godina dijagnostike



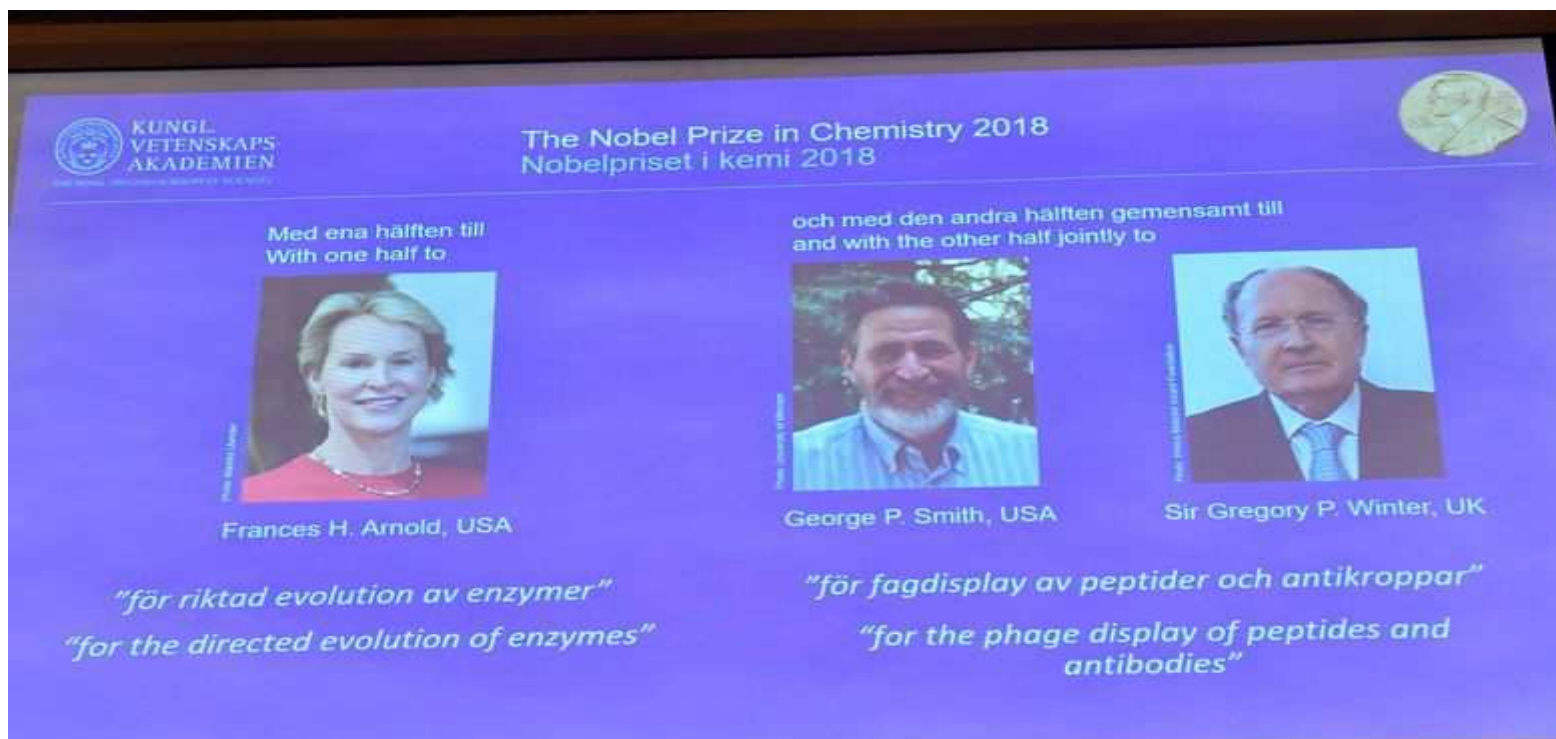


Nanotehnologije



Nobelova nagrada za kemiju 2016.

Biotehnologija i medicina



The slide is a purple background with white and yellow text. At the top left is the logo of the Royal Swedish Academy of Sciences (Kungl. Vetenskapsakademien). At the top right is a gold Nobel Prize medal. The title 'The Nobel Prize in Chemistry 2018' and 'Nobelpriset i kemi 2018' is centered at the top. Below the title, the text 'Med ena hälften till' and 'With one half to' is on the left, and 'och med den andra hälften gemensamt till' and 'and with the other half jointly to' is on the right. Three portraits are shown: Frances H. Arnold (left), George P. Smith (middle), and Sir Gregory P. Winter (right). Below each portrait is their name and nationality. At the bottom, the award reasons are given in Swedish and English.

KUNGL. VETENSKAPSKAS AKADEMIEN

The Nobel Prize in Chemistry 2018
Nobelpriset i kemi 2018

Med ena hälften till
With one half to

och med den andra hälften gemensamt till
and with the other half jointly to

Frances H. Arnold, USA

George P. Smith, USA

Sir Gregory P. Winter, UK

"för riktad evolution av enzymer"
"for the directed evolution of enzymes"

"för fagdisplay av peptider och antikroppar"
"for the phage display of peptides and antibodies"

Nobelova nagrada za kemiju 2018.

Prošlost - sadašnjost –budućnost ?



Hvala za pozornost!

Jadranka Sertić

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