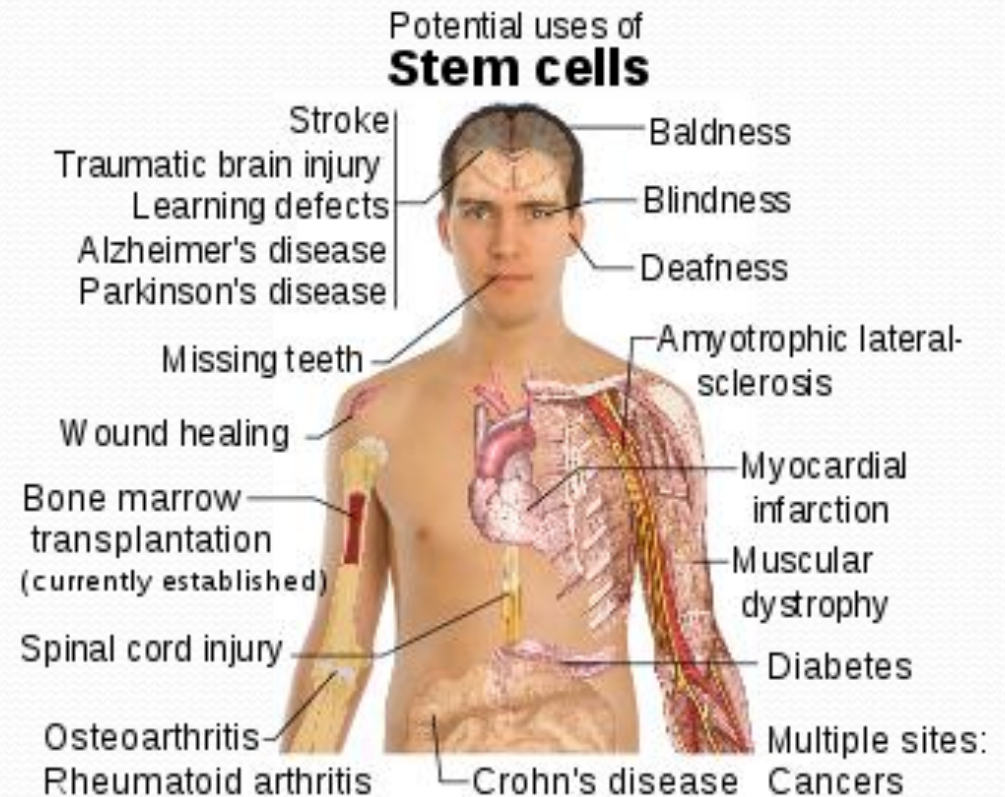


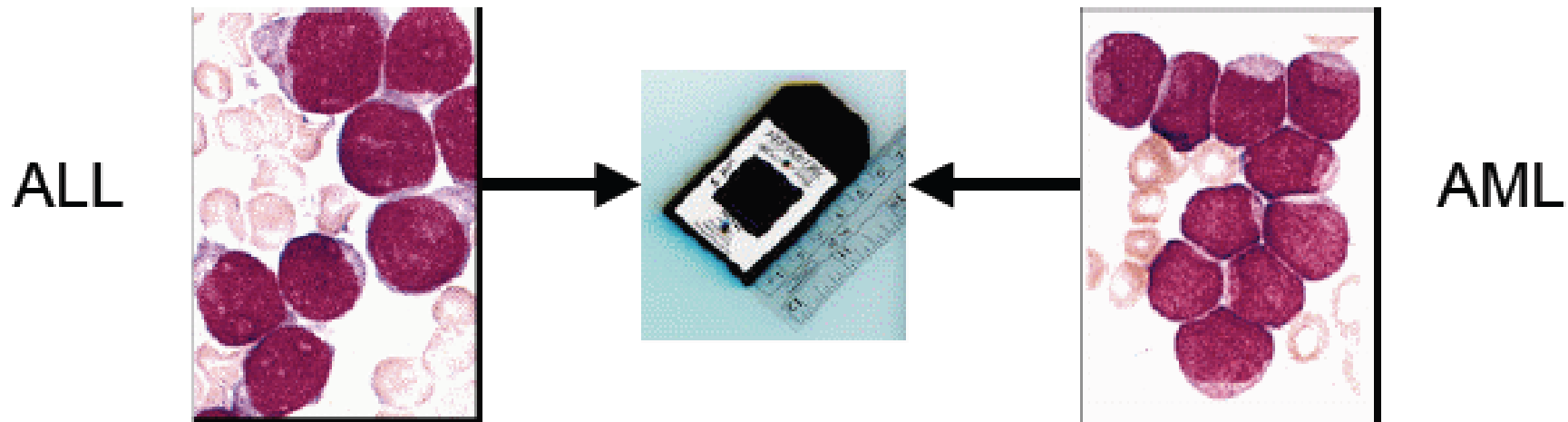
# Stem Cells

- Stem cells are currently being tested to treat everything from Crohn's disease to baldness!
- The main areas where stem cells have proven their worth is in bone marrow transplants, replacing damaged heart tissue after a heart attack and replacing damaged nerve tissue which gives hope to anyone who has had a spinal cord injury.



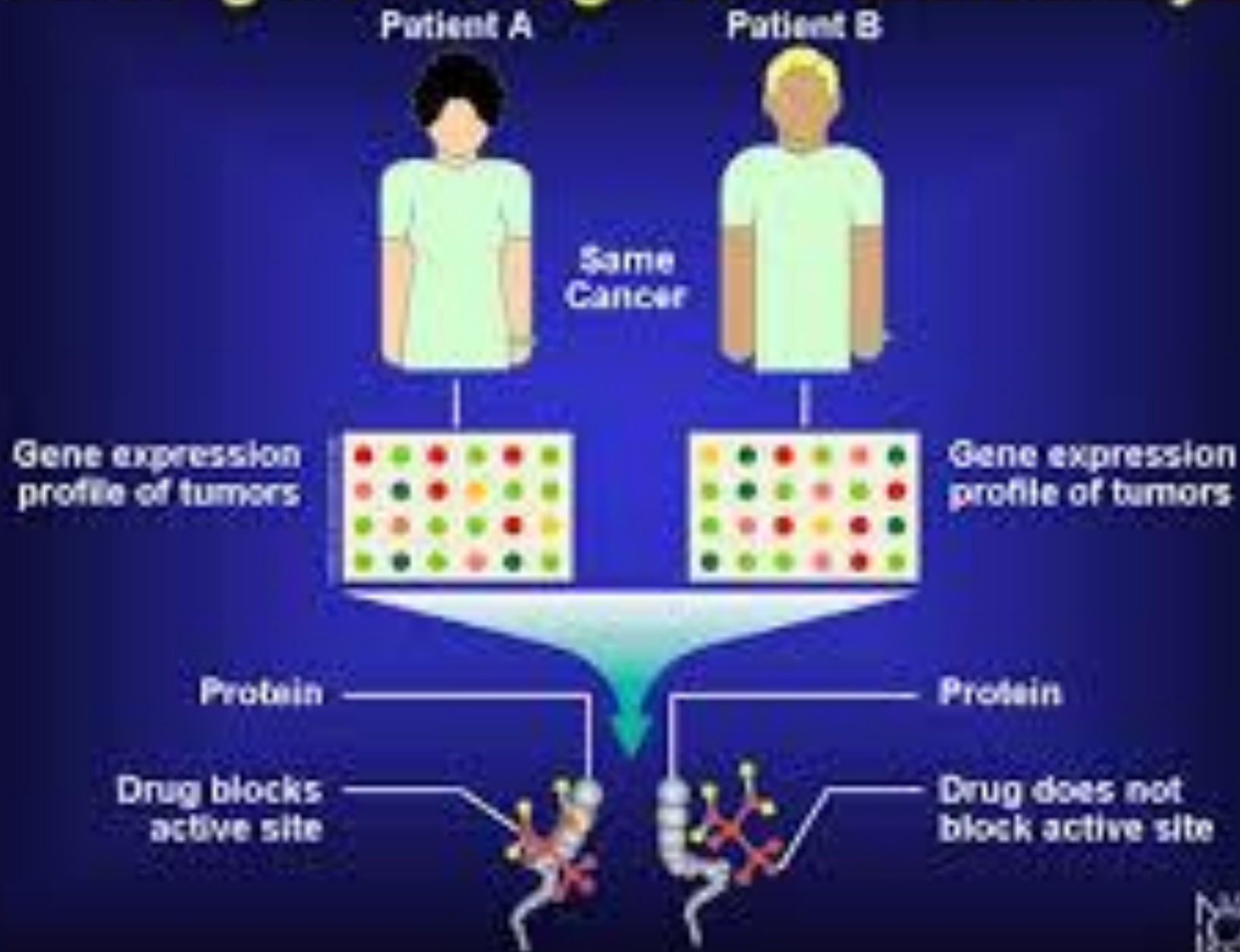
# Microarrays: An Example

- Leukemia: Acute Lymphoblastic (ALL) vs Acute Myeloid (AML), Golub et al, Science, v.286, 1999
  - 72 examples (38 train, 34 test), about 7,000 genes
  - well-studied (CAMDA-2000), good test example

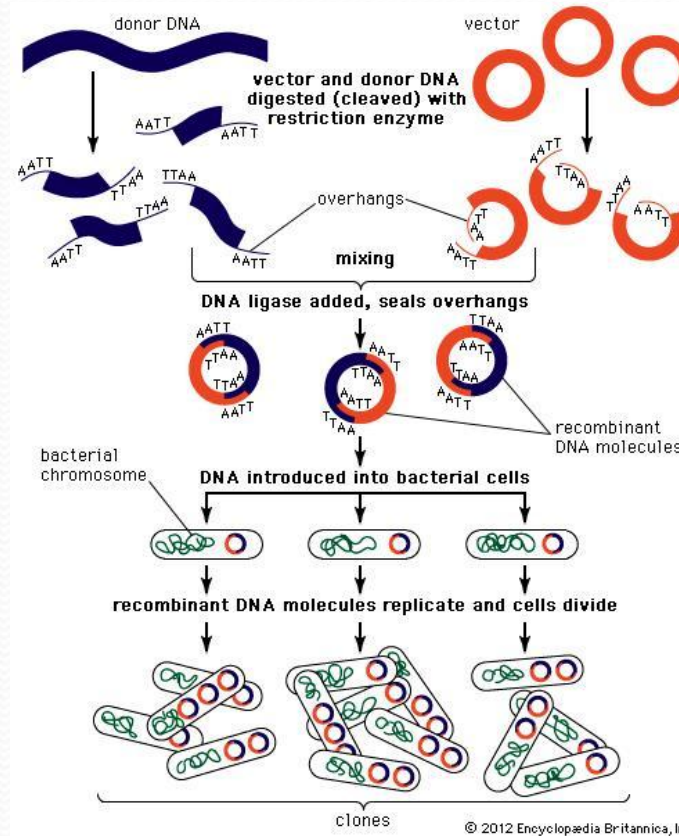


Visually similar, but genetically very different

# Finding New Drugs With Microarrays



# Recombinant Hormones



# Bioprocessing

- The main product currently bioprocessed is insulin, the human protein responsible for lowering blood sugar after eating.
- The human gene for insulin is placed into bacteria, these are cultured and allowed to produce insulin which is collected, purified and sold to the millions of diabetics worldwide.



# Therapeutic proteins

✓ High specificity and less toxicity → high safety and efficacy

✓ Therapeutic proteins

- Antibodies, proteins, enzymes, peptides etc.

ex) EPO, Interferon, Insulin, Avastin, Enbrel, Remicade, Herceptin,

EPO (Erythropoietin) : Stimulating the proliferation of red blood cells

Herceptin : Mab against EGFR2(Epidermal growth factor receptor 2)

Avastin : Mab against VEGF (Vascular endothelial growth factor)

Remicade: Mab against TNF- $\alpha$  (**Tumor necrosis factor-  $\alpha$** )

✓ World market

- EPO alone : ~ \$ 11 Billion per year

- \$ 50 Billion (2007) → \$ 190 Billion (2015)

- Antibodies > 50 %

- Intensive investment in monoclonal antibodies: Biosimilar

**Therapeutic proteins will form the back-bone of future medicinal therapy**

# Therapeutic Enzymes

Disease	Product	Developer	Sales (US\$Millions)		Features
			2004	2007	
Gaucher's	Ceredase®	Genzyme	443	N/A	<ul style="list-style-type: none"> <li>▪ Glucocerebrosidase</li> <li>▪ Purified from human placenta</li> </ul>
	Cerezyme®	Genzyme	932 (2005)	1,048	<ul style="list-style-type: none"> <li>▪ Produced in CHO cells</li> <li>▪ 3 Exoglycosidases process for Terminal Mannose</li> </ul>
Fabry's	Fabrazyme®	Genzyme	209	397	<ul style="list-style-type: none"> <li>▪ alpha-galactosidase</li> <li>▪ Mannose-6-phosphate for Glycotargeting</li> </ul>
	Replagal	TKT	57	168	
MPS-1	Aldurazyme®	Genzyme	12	204	<ul style="list-style-type: none"> <li>▪ alpha -L-iduronidase</li> </ul>
Pompe	Myozyme®	Genzyme	Approved (2006)		<ul style="list-style-type: none"> <li>▪ alfa-glucosidase</li> </ul>

Treatment of Gaucher's disease by Cerezyme costs up to \$550,000 annually

Most of therapeutic enzymes : glycoproteins

# Tissue Engineering





# Tissue Engineering

- Tissue engineers have created artificial skin, cartilage and bone marrow.
- Current projects being undertaken include creating an artificial liver, pancreas and bladder.
- Again, we are far from replacing a whole organ, but just looking for “refurbishing” our slightly used ones at the moment.

