HEALTH CARE MODEL IN A TRANSITION ECONOMY: Croatian model of financing rare diseases

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Are rare diseases a priority for Central / Eastern European Public Health?

RARE DISEASES

- AROUND 1-2% OF ALL NEWBORNS HAVE SOME OF INHERITED METABOLIC DISORDERS
- PREVALENCE LESS THAN 5 in 10.000 NEWBORNS
- PROGRESSIVE AND FATAL OUTCOME IN MOST OF THEM WHEN DIAGNOSIS WAS NOT ESTABLISHED IN TIME
- VERY HIGH RISK OF RECURRENCE IN FAMILIES
- THERE IS EFFECTIVE TREATMENT FOR SOME OF THIS DISEASES

RARE DISEASES

- IT IS ONLY A MATTER OF GENETICS

WHAT DOES IT MEAN FOR HEALTH CARE SYSTEM?

- NEW DIAGNOSTIC TOOLS
- NEW THERAPEUTIC METHODS
- NEW GROUP OF DISEASES
- COST

WHAT DOES IT MEAN FOR THE POPULATION?

- PATHOGENESIS OF DISORDERS
- POPULATION GENETICS
- SCREENING METHODS

WHAT DOES IT MEAN FOR INDIVIDUAL PERSON?

- Good quality of life if diagnosis is established in time for some of diseases
- Enzyme Replacement Therapy (ERT)
- Gene therapy

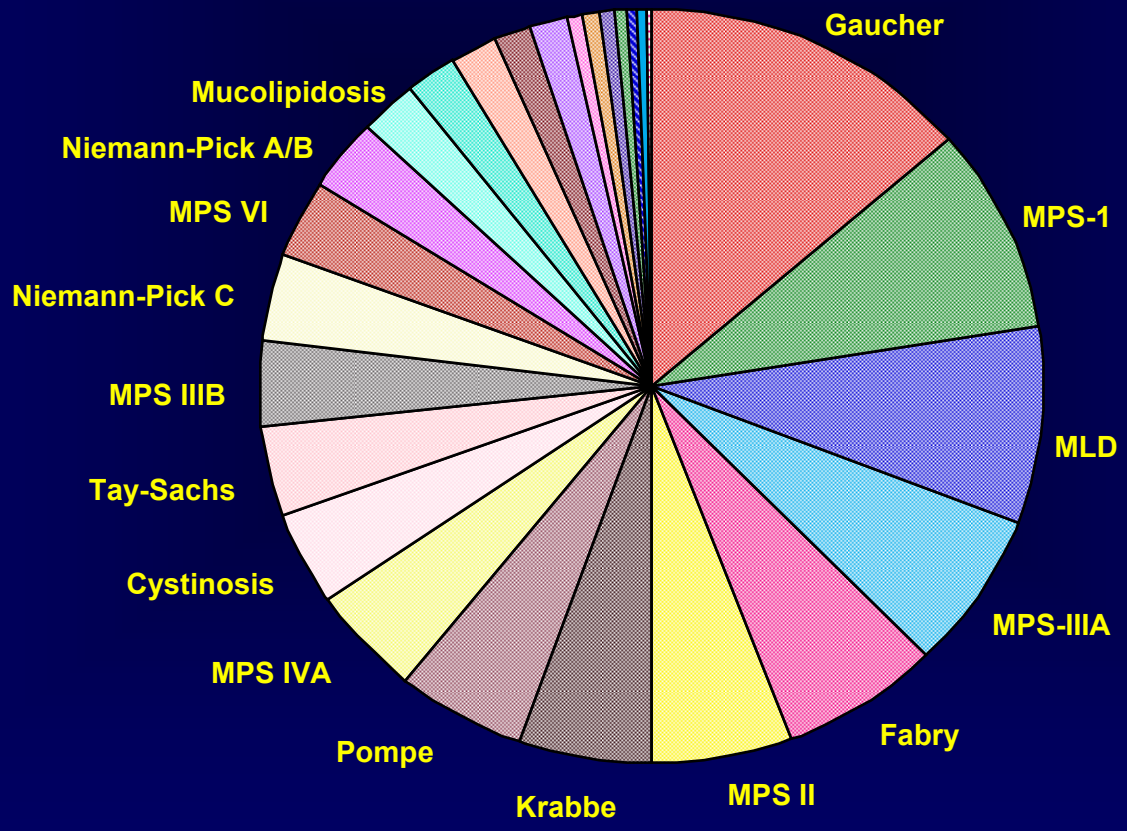
WHAT LYSOSOMAL STORAGE DISEASES ARE?

- Accumulation of substrate within the lysosome usually due to deficiency of a catabolic enzyme

- ✓ POPULATION SIZE
- ✓ APPROPRIATE CURE
- ✓ HOW TO FUND THE COST OF TREATMENT

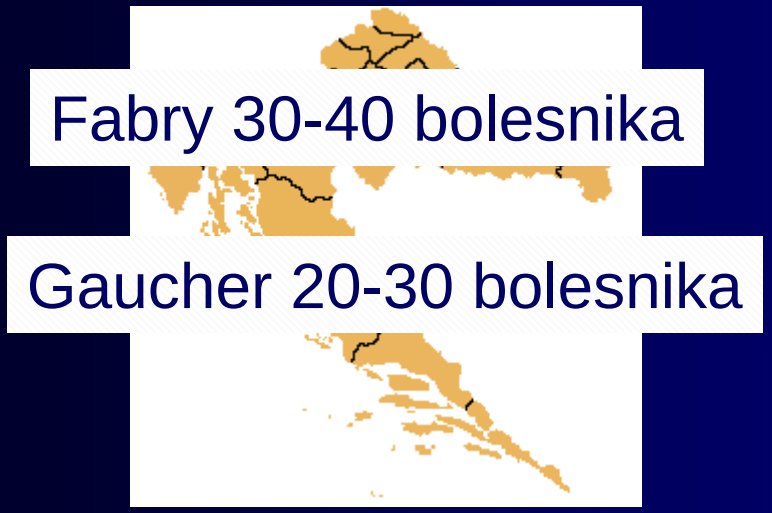
LSD has no ethnic predilection

Propensity of Lysosomal Storage Disorders

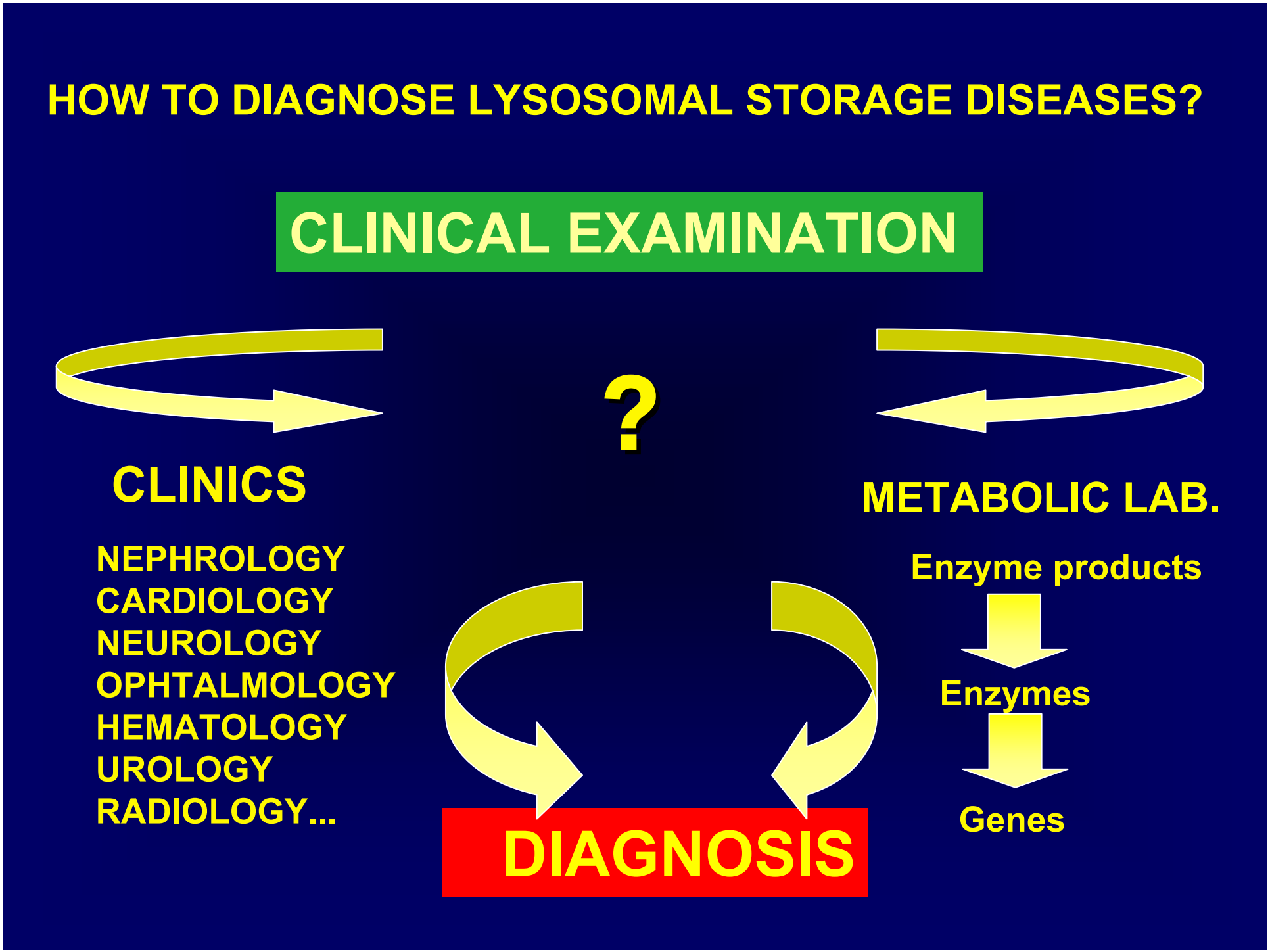


LYSOSOMAL STORAGE DISEASES ZAGREB EXPERIENCE 1994. - 2005.

MPS I	7
MPS II	4
MPS IIIa	3
MPS IVa	5
MPS VI	6
M.Fabry	1
M.Gaucher	11
M.Farber	1



HOW TO DIAGNOSE LYSOSOMAL STORAGE DISEASES?



LABORATORY DIAGNOSTIC PROCEDURES

SERA

- MUKOLIPIDOSIS II I III
- SFINGOLIPIDOSIS (M.Gaucher)-citotriozidaza,ACE

LEUKOCYTES

- ALL LYSOSOMAL HYDROLASES
  except:
- mukolipidosis I
- α-glukozidasis (M.Pompe)
- fosfotransferasis (ML II i III)

FIBROBLASTS

CONFIRMATION OF LYSOSOMAL STORAGE DISEASE DIAGNOSIS

- ✓ POPULATION SIZE
- ✓ APPROPRIATE CURE
- ✓ HOW TO FUND THE COST OF TREATMENT

CURRENT AVAILABLE TREATMENT

- ✓ENZYME REPLACEMENT THERAPY – IV ROUTE
- ✓ENZYME REPLACEMENT THERAPY – ORAL ROUTE
- ✓HEMATOPOIETIC STEM CELL TRANSPLANTATION

COST OF TREATMENT

- ✓ ERT ~150 000 EUR/year/patient
- ✓ BMT ~ 100-150 000 EUR/patient

- ✓ ERT IS EXPENSIVE !
- ✓ BMT IS LESS EXPENSIVE !
- ✓ WHAT'S ABOUT CURABILITY ?

- ✓ POPULATION SIZE
- ✓ APPROPRIATE CURE
- ✓ HOW TO FUND THE COST OF TREATMENT

CONSTITUTIONAL LAW

- ✓ Every citizen of Croatia should have equal rights to obtain appropriate health care

RARE DISEASES

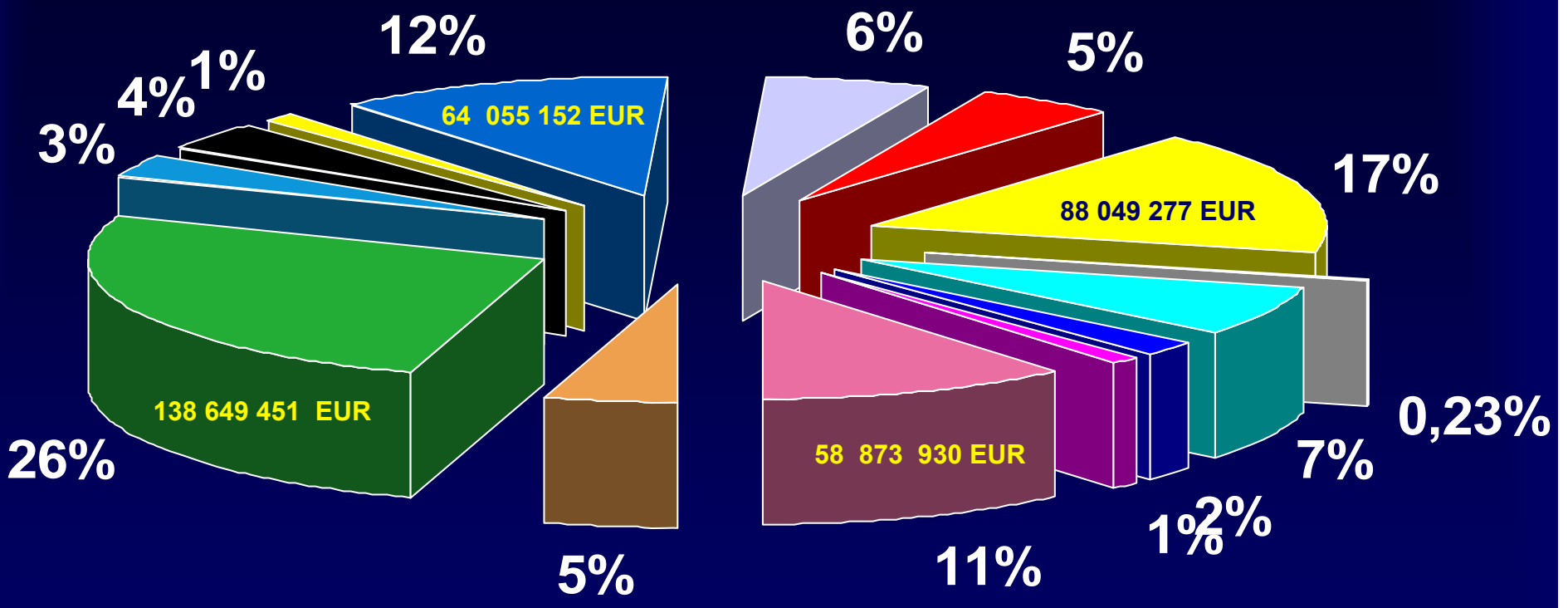
- IT IS ONLY A MATTER OF GENETICS

COST OF THE TREATMENT (CROATIA)

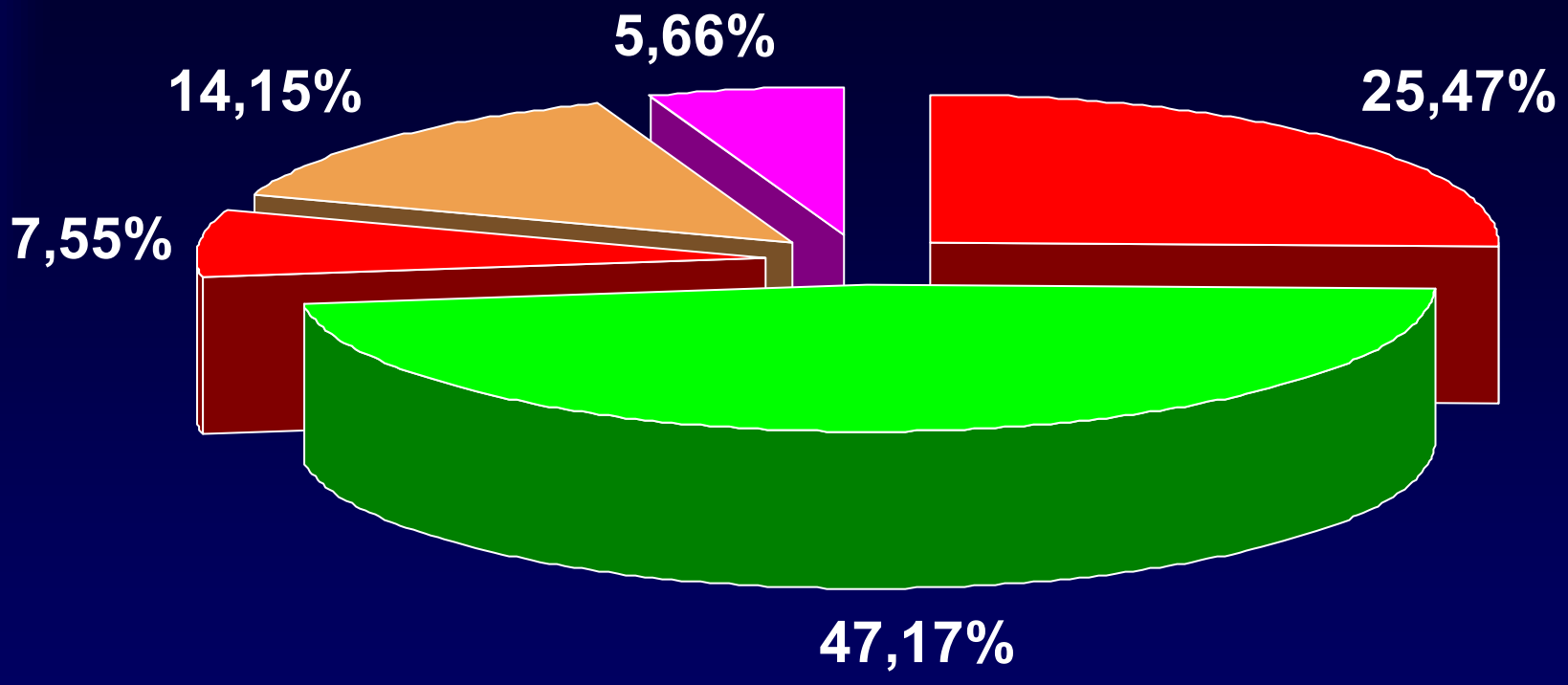
- ✓ Gaucher disease (9 pts)
  - 1 350 000 EUR/year
- ✓ Fabry disease (1 pt)
  - 180 000 EUR/year/
- ✓ MPS I (3 pt)
  - 480 000 EUR/year/

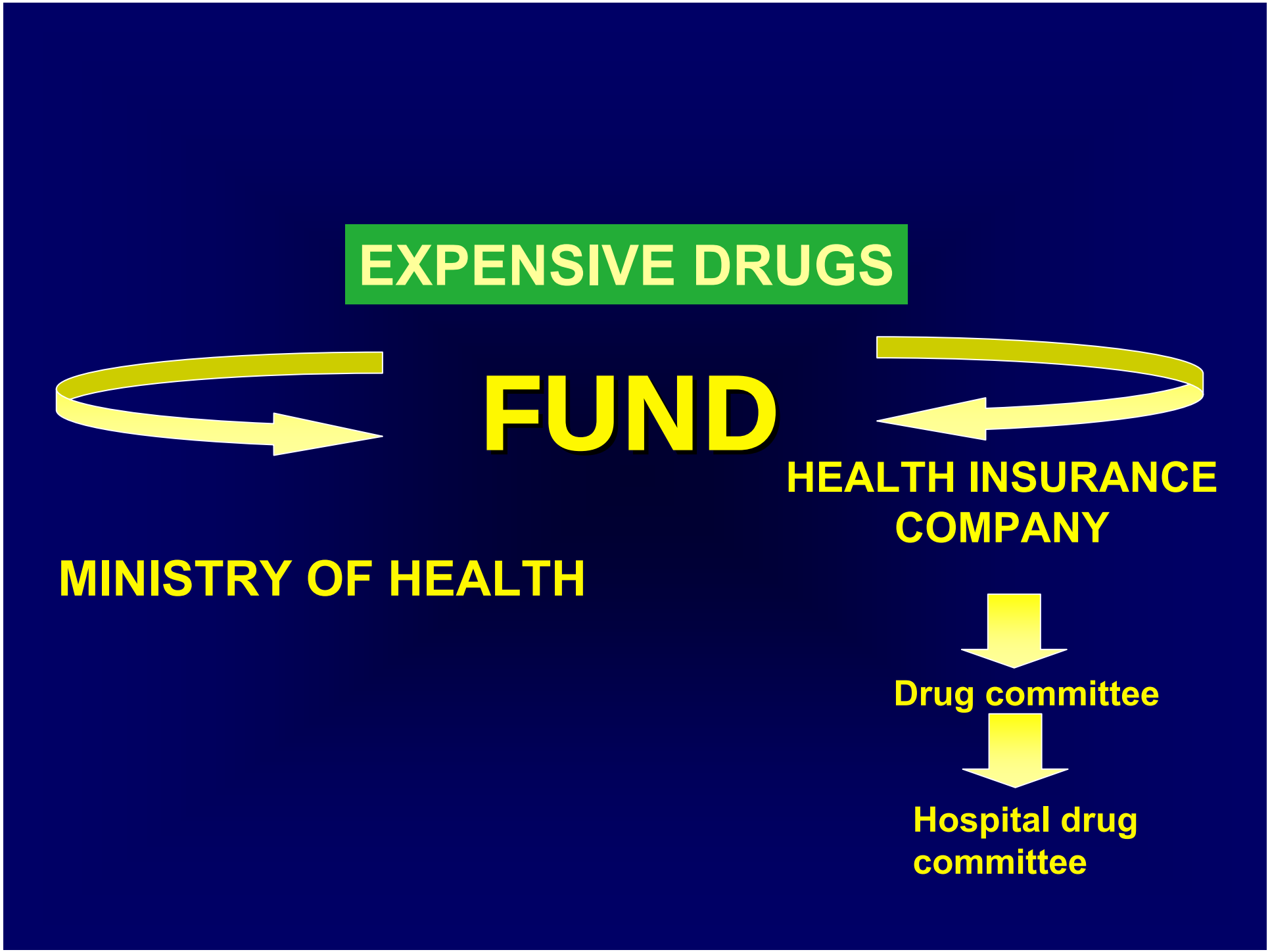
Total cost of enzyme replacement therapy = 2 010 000 EUR

Total drug consumption in Croatia (2004)



COST OF DISABILITY
(2 100 000 ∈)




EXPENSIVE DRUGS
FUND
HEALTH INSURANCE COMPANY
MINISTRY OF HEALTH
Drug committee
Hospital drug committee

FUND

- The main goal is to offer same and equal treatment to every citizen of Croatia suffering of rare disease or a disease that require expensive or/and prolonged treatment.

Hospital funding 2002/2003

Total hospital budget

Cost of hospital beds

Transplantation

Diagnostic procedures

Dialysis

Outpatient hospital cost

Prepaid procedures (eg. hip replacement, arthroscopy, etc)

Expensive therapy

FUND 2002

- Haemophilia and other congenital bleeding disorders
- AIDS
- Adenosin-deminase deficiency
- Inherited metabolic disorders
- Multiple sclerosis
- Chronic myeloid leukaemia
- Juvenile arthritis
- Ovarian cancer

FUND 2003

- Haemophilia and other congenital bleeding disorders
- AIDS (five drugs regimen)
- Resistant tuberculosis (antituberculotics)
- Adenosin-deminase deficiency (recombinant adenosin deaminase)
- Inherited metabolic disorders (Gaucher, Fabry, MPS I)
- Multiple sclerosis (beta interferons)
- Chronic myeloid leukaemia (imatinib)
- Chronic lymphoid leukemia (fludarabine)
- Juvenile arthritis (monoclonal antibody)
- Ovarian cancer (taxanes)
- Breast cancer (monoclonal antibody, taxanes)
- Non Hodgkin lymphoma (monoclonal antibody)
- Pancreatic cancer (gemcitabine)
- Colon cancer (irinotecan)
- Prostate cancer (LH-RH antagfonists)
- Growth retardation (recombinant growth hormons)

Hospital funding 2005

Funding of LSD, haemophilia and ADA deficiency

- Total hospital budget
- Cost of hospital beds
- Transplantation
- Diagnostic procedures
- Dialysis
- Outpatient hospital cost
- Prepaid procedures (eg. hip replacement, arthroscopy, etc)
- Expensive therapy

FUND 2005

Direct funding

- Haemophilia and other congenital bleeding disorders
- Adenosin-deminase deficiency (recombinant adenosin deaminase)
- Inherited metabolic disorders (Gaucher, Fabry, MPS I)

Funding through hospital budget

- AIDS (five drugs regimen)
- Resistant tuberculosis (antituberculotics)
- Multiple sclerosis (beta interferons)
- Chronic myeloid leukaemia (imatinib)
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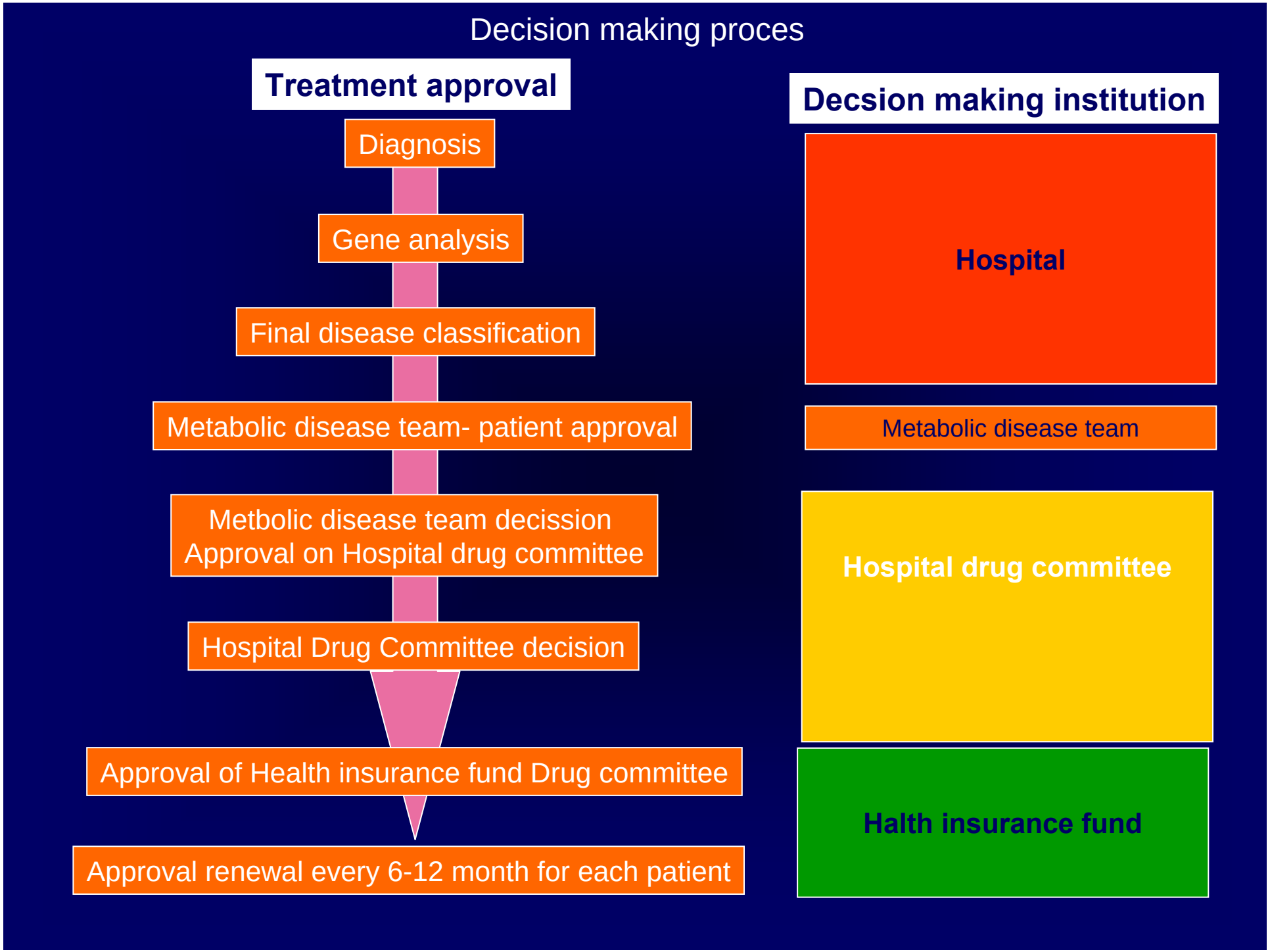
Decision making proces
Treatment approval
Diagnosis
Gene analysis
Final disease classification
Metabolic disease team- patient approval
Metbolic disease team decission
Approval on Hospital drug committee
Hospital Drug Committee decision
Approval of Health insurance fund Drug committee
Approval renewal every 6-12 month for each patient
Decsion making institution
Hospital
Metabolic disease team
Hospital drug committee
Halth insurance fund

FUND

- All patients have a **equal chance** to be treated
- All patients have **equal treatment**
- Resources and **treatment control**