

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**

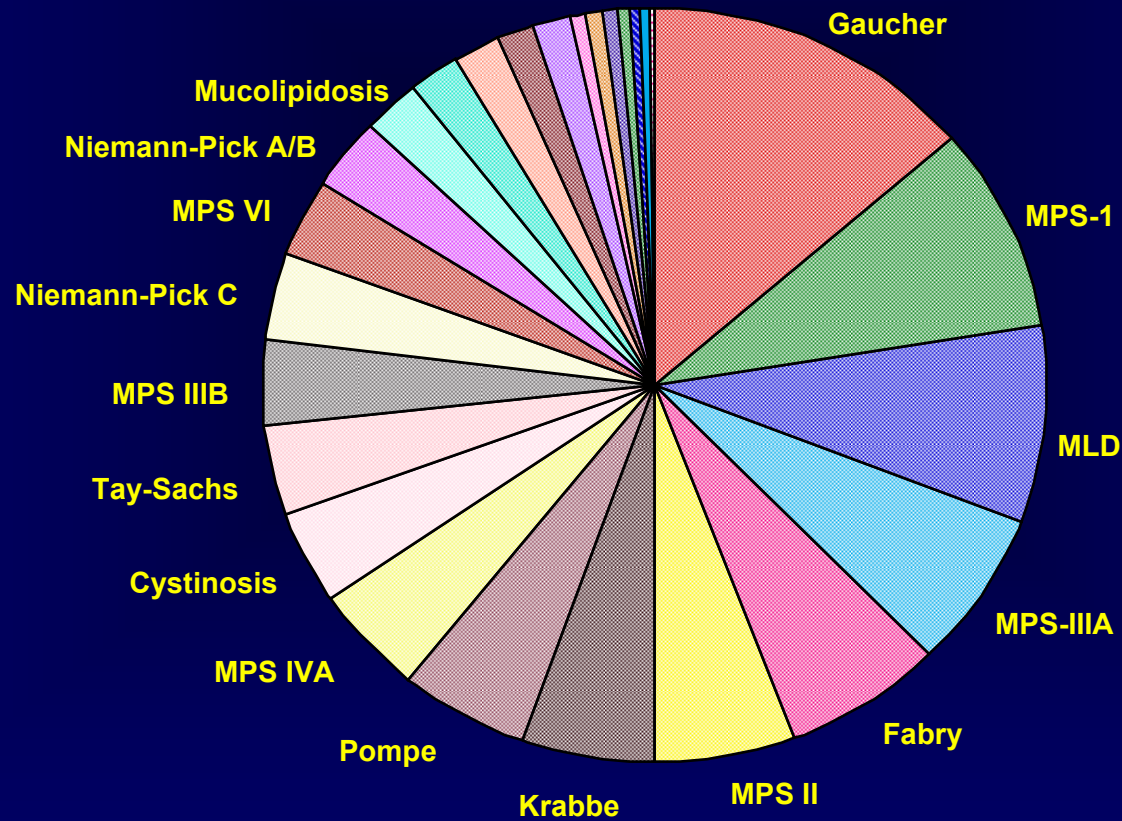
✓ **APPROPRIATE CURE**

✓ **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

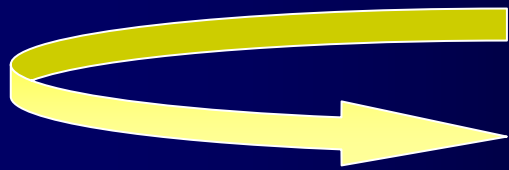


Fabry 30-40 bolesnika

Gaucher 20-30 bolesnika

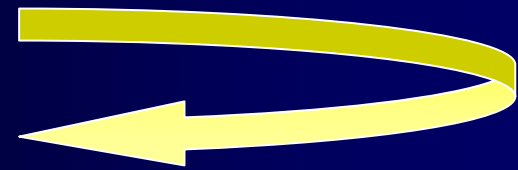
HOW TO DIAGNOSE LYSOSOMAL STORAGE DISEASES?

CLINICAL EXAMINATION



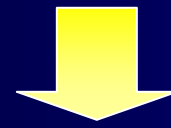
CLINICS

- NEPHROLOGY
- CARDIOLOGY
- NEUROLOGY
- OPHTHALMOLOGY
- HEMATOLOGY
- UROLOGY
- RADIOLOGY...



METABOLIC LAB.

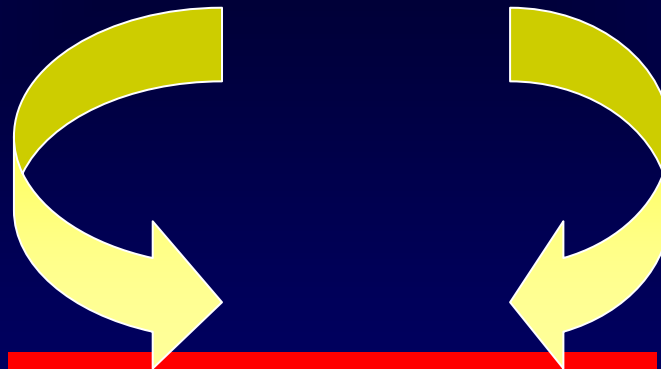
Enzyme products



Enzymes



Genes



DIAGNOSIS

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

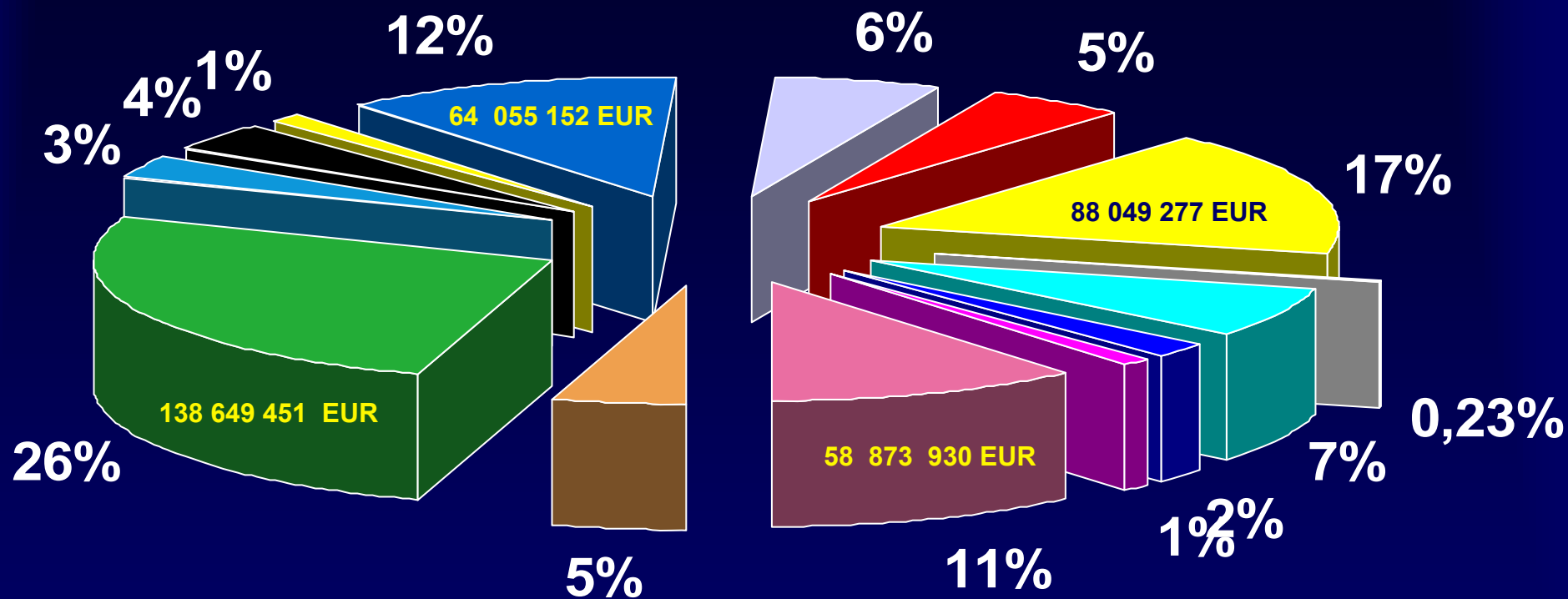
- **IT IS ONLY A MATTER OF GENETICS**

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/**

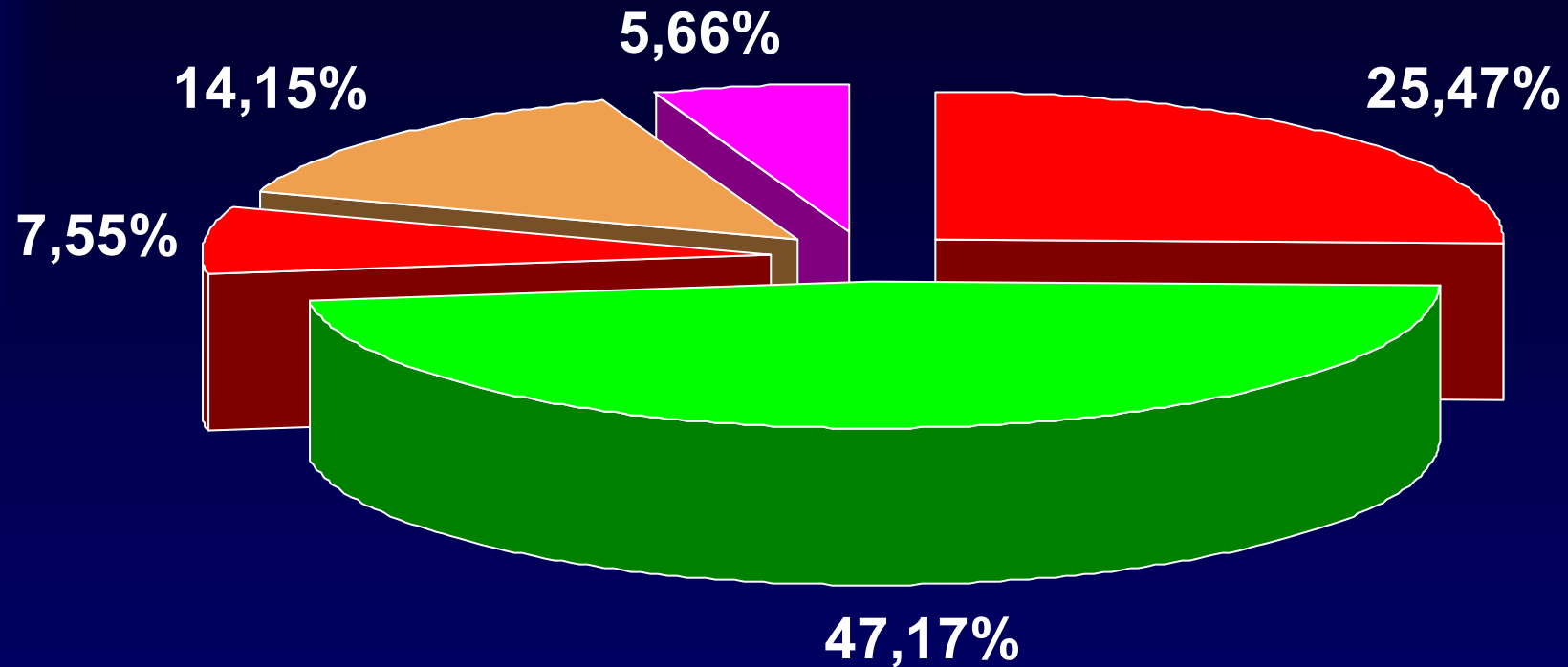
Total cost of enzyme replacement therapy = 2 010 000 EUR

Total drug consumption in Croatia (2004)



- Group A
- Group B
- Group C
- Group D
- Group E
- Group F
- Group G
- Group H
- Group I
- Group J
- Group K
- Group L
- Group M
- Group N
- ERT
- Group O
- Group P
- Group Q
- Group R
- Group S
- Other

COST OF DISABILITY (2 100 000 €)



■ Net loss

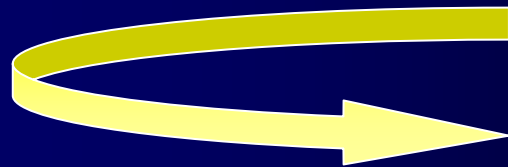
■ Tax loss

■ Sicknes leave

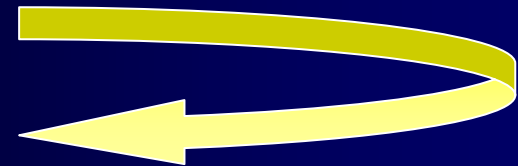
■ Hospitalisation cost

■ Insurance cost

EXPENSIVE DRUGS

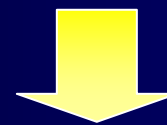


FUND



**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 hormones)

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 (antituberculosics)
- ❖ Multiple sclerosis (beta interferons)
- ❖ Chronic myeloid leukaemia (imatinib)
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- ❖ Juvenile arthritis (monoclonal antibody)
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- ❖ Growth retardation (recombinant growth hormones)

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**



