

THE HEALTH-ECONOMIC EVALUATION OF RISDIPLAM IN PATIENTS WITH SPINAL MUSCULAR ATROPHY

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OBJECTIVES

- Spinal muscular atrophies (SMA) are clinically and genetically heterogenous inherited orphan diseases leading to progressive spinal motoneurons degeneration and loss of function.
- There are 4 types of SMA from which type I is the most severe. SMA patients are need during the whole life in lots of services (medical, social etc.) and the cost of the care has never been calculated in Russia before.
- According to Russian register in the middle of 2020 there were **998** patients with SMA in Russia.
- 78%** of patients in the register have Type II and III SMA, Type I SMA have **21%** of patients and SMA IV type – less than **1%**.
- The two pathogenetic therapies are authorized in Russia: risdiplam and nusinersen.
- The **aim of the study** was to perform health-economic evaluation of risdiplam.

MATERIALS AND METHODS

- The study was done from the **state healthcare perspective**.
- The studied indication was **spinal muscular atrophy**.
- There was only **one comparator** – nusinersen.
- The choice of the method health-economic analysis:
 - ✓ No head to head comparison of risdiplam and nusinersen
 - ✓ No indirect including network comparison of risdiplam and nusinersen
 - ✓ Assumption of equivalent efficacy

cost-minimizing analysis

- Direct medical costs:**
 - ✓ SMA pathogenic therapy (risdiplam and nusinersen cost)
 - ✓ Cost of drug injection
 - ✓ Cost of adverse events treatment
- Sources of costs:**
 - ✓ Governmental drugs price register
 - ✓ Disease related groups costs
 - ✓ The program of healthcare guarantee

RESULTS

- Direct medical costs of risdiplam and nusinersen are shown in Fig. 1.

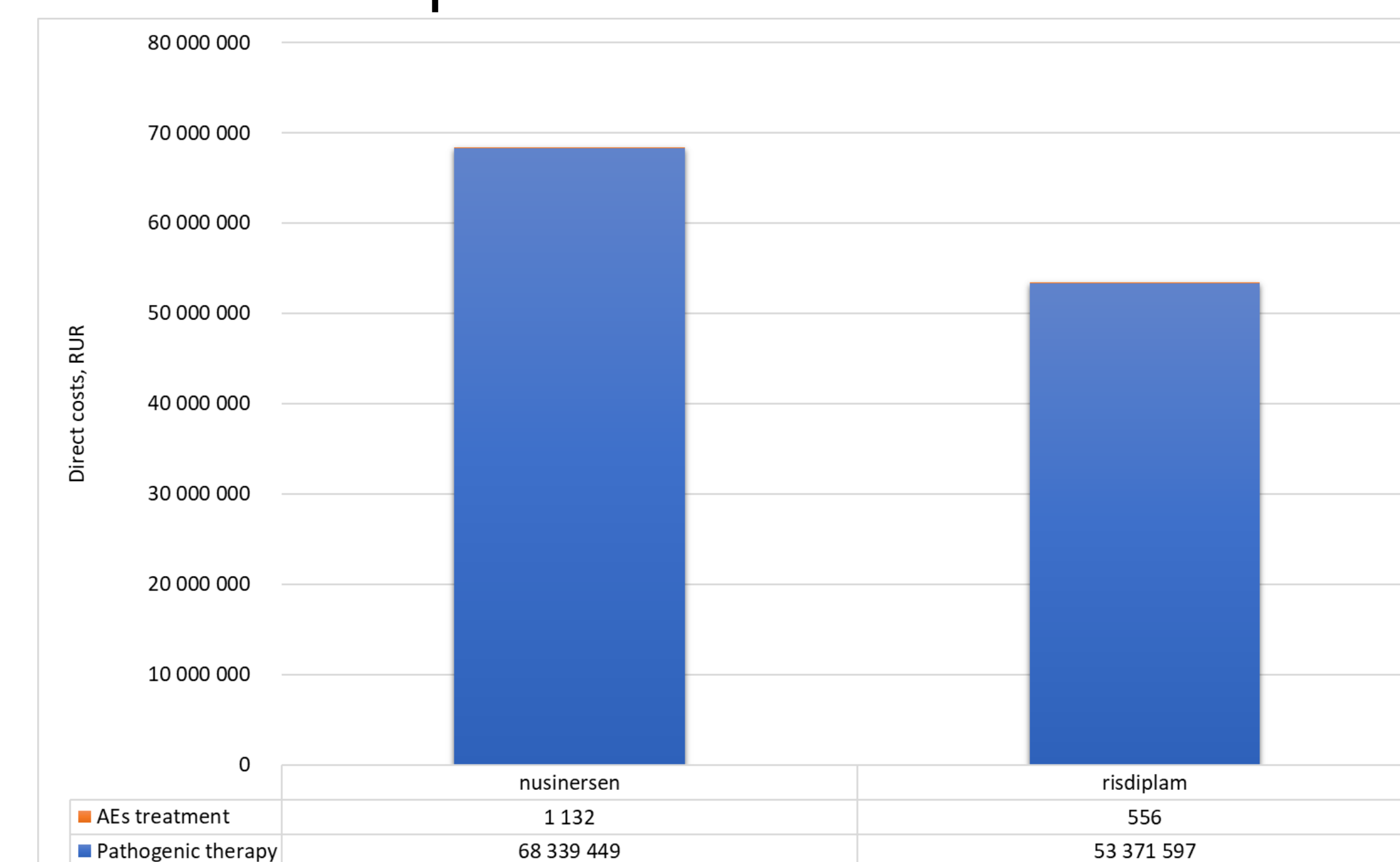


Figure 1.

- In direct medical costs structure **more than 99%** of costs were risdiplam or nusinersen cost.
- Cost-minimizing ratio is shown in the *Table* below.

Drug	Total direct medical costs, RUR	Cost-minimizing ratio, RUR
Nusinersen	67 830 718	-
Risdiplam	51 817 084	- 16 014 193,39

- The results of budget impact analysis are shown in Fig. 2.

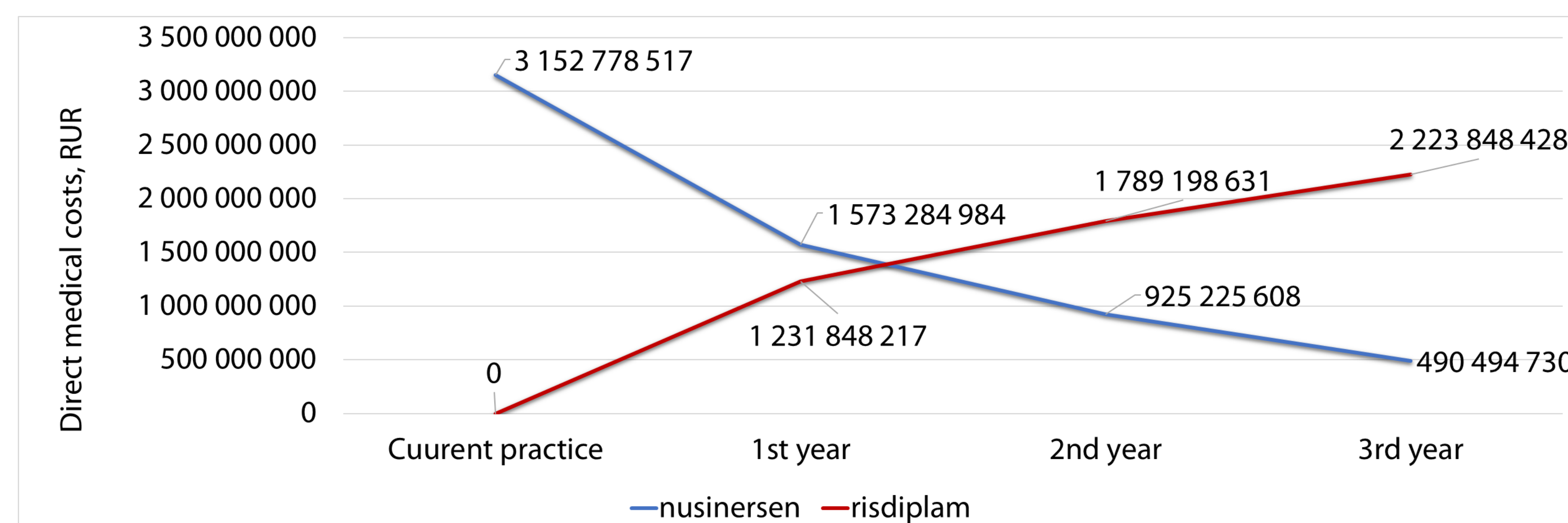


Figure 2.

CONCLUSION

- ✓ The direct medical costs of **risdiplam** are **by 23,6% lower** comparing to nusinersen
- ✓ The growth of risdiplam share from **0% to 8,5%** during three years lead to **18,8%** budget economy