

# ECONOMIC BURDEN OF PRIMARY BILIARY CHOLANGITIS (PBC) IN SPAIN

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## INTRODUCTION

- Primary Biliary Cholangitis (PBC) is a rare, chronic, cholestatic, autoimmune liver disease, characterised by progressive destruction of the small intrahepatic bile ducts, leading to fibrosis, cirrhosis, and liver failure if inappropriately treated<sup>1</sup>.

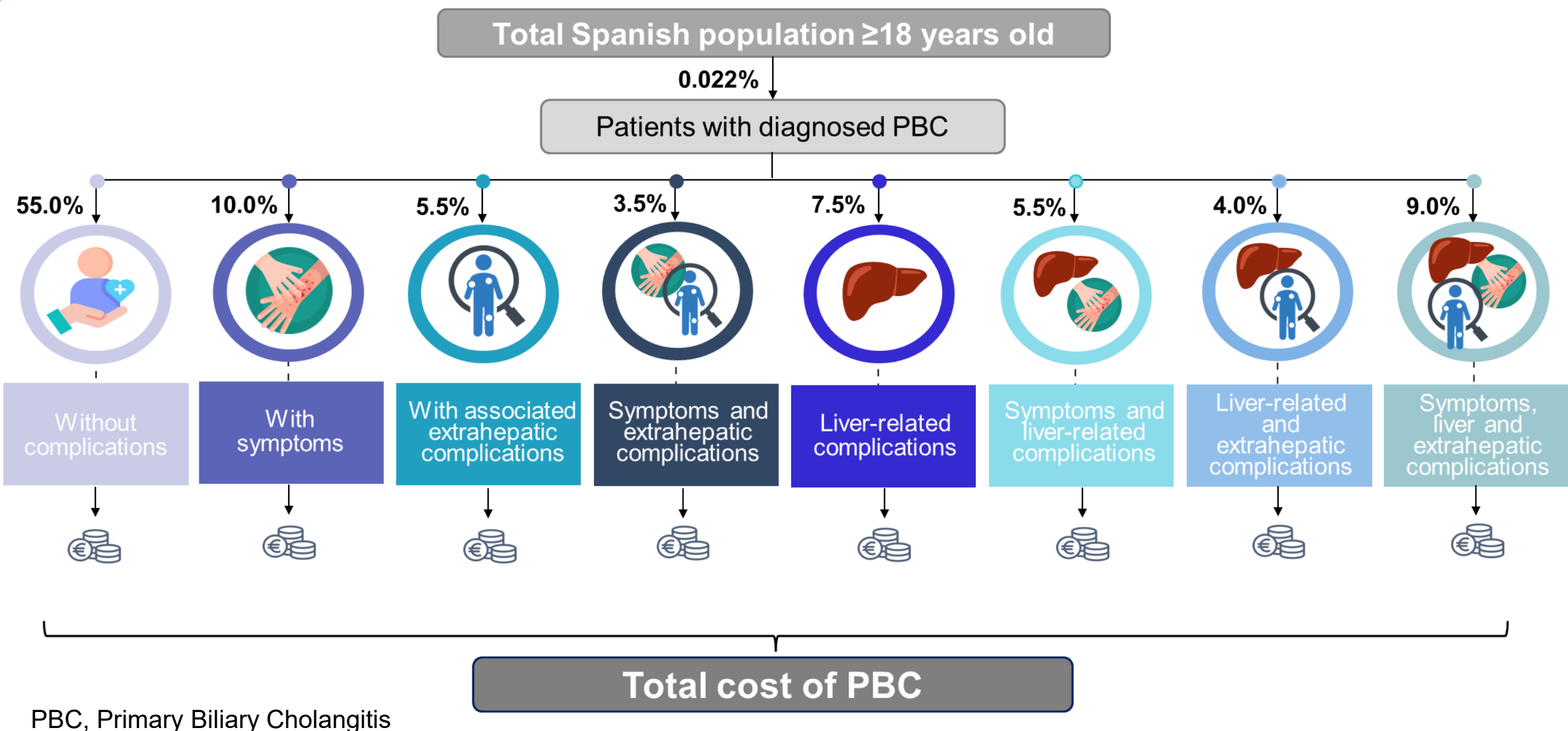
## OBJECTIVE

The aim of this analysis was to estimate the economic burden of managing adult patients with PBC in Spain

## MATERIALS AND METHODS

- A cost analysis model was developed to estimate the economic burden of managing patients diagnosed with PBC in Spain over a 1-year time horizon, based on data extracted from the literature and validated by a panel of experts.
- The target population was calculated based on the Spanish general population ≥18 years<sup>2</sup> and the prevalence of diagnosed PBC in Spain, estimated from national epidemiological data available in the literature<sup>3</sup>.
- Patients diagnosed with PBC were distributed based on whether they presented one or more of the following clinical situations: without complications, with symptoms, with associated extrahepatic complications, and/or with liver-related complications. The proportion of patients for each situation was established based on data reported by the expert panel (Figure 1).

Figure 1. Model structure

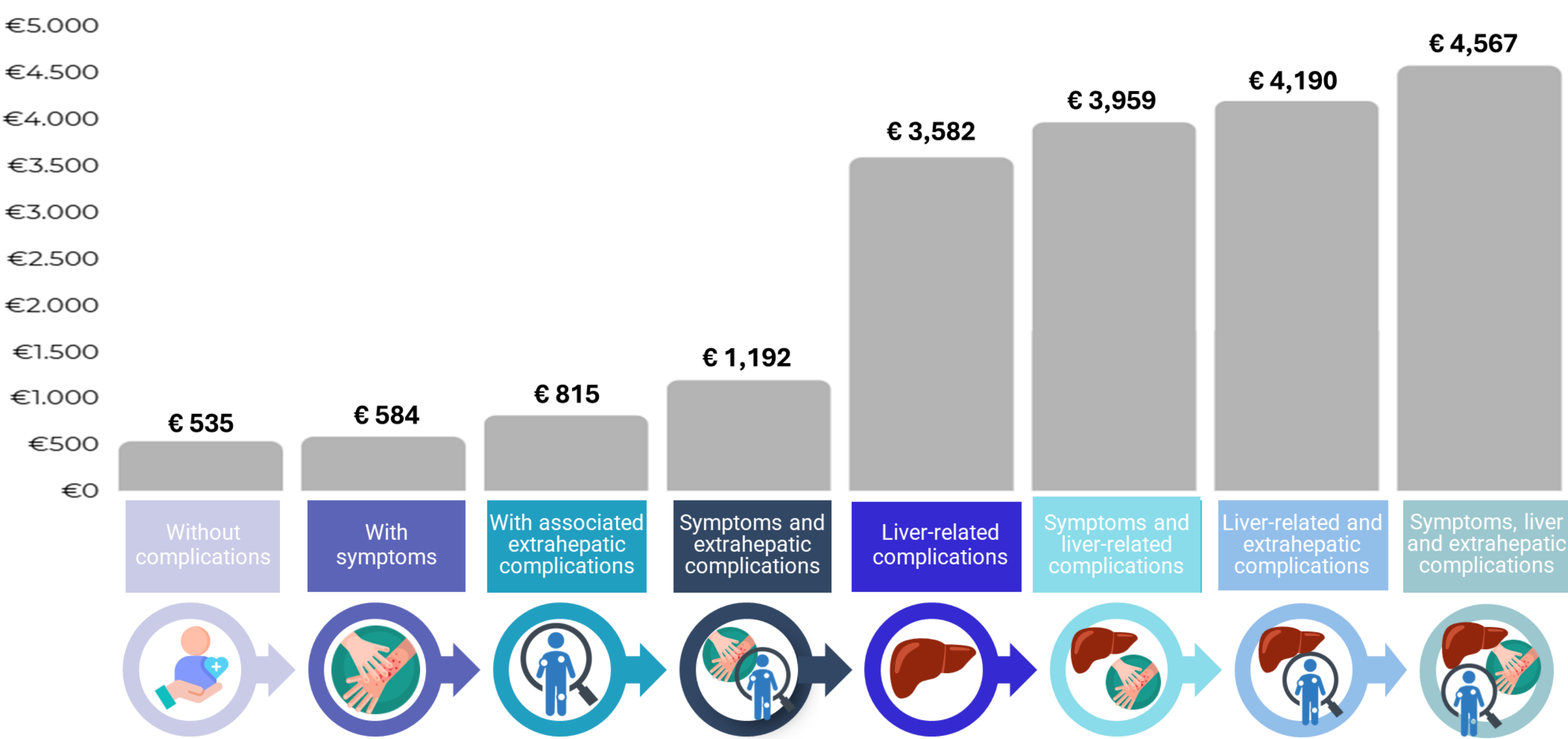


- Additionally, within each situation, clinically and economically relevant symptoms and complications that PBC patients may experience were considered based on data reported in the literature<sup>3-11</sup>. For patients without complications, only the management of PBC was considered. For symptoms, pruritus and fatigue were included; associated extrahepatic complications included osteoporosis, hyperlipidaemia, fat-soluble vitamin malabsorption, and coexisting autoimmune diseases (Sjögren's syndrome, hypothyroidism, rheumatoid arthritis, scleroderma, and Raynaud's syndrome); and for liver-related complications, compensated cirrhosis without portal hypertension, compensated cirrhosis with portal hypertension, decompensated cirrhosis, hepatocellular carcinoma, and liver transplantation were considered.
- The National Health System was selected, meaning, direct healthcare costs were included, comprising drug treatment costs and costs associated with the management of the disease, symptoms and complications.
- For treatment costs, the analysis assumed that 69% of patients were treated with ursodeoxycholic acid (UDCA), 30% received UDCA combined with bezafibrate, and 1% were enrolled in a clinical trial, based on data provided by the expert panel reflecting routine clinical practice.
- Management costs were determined through healthcare resources consumption defined by clinical experts in the hepatology field. Unitary costs (€, 2024) were derived from local databases<sup>12</sup> and the literature<sup>13</sup>.
- Sensitivity analyses (SA) were performed to identify the parameters with the greatest impact and assess their influence on the results by individually varying the following inputs: proportion of PBC patients with symptoms<sup>3</sup>, and with associated extrahepatic complications<sup>3</sup>; liver complications and transplantations costs<sup>14</sup>; and annual liver transplantation rate<sup>15</sup>

## RESULTS

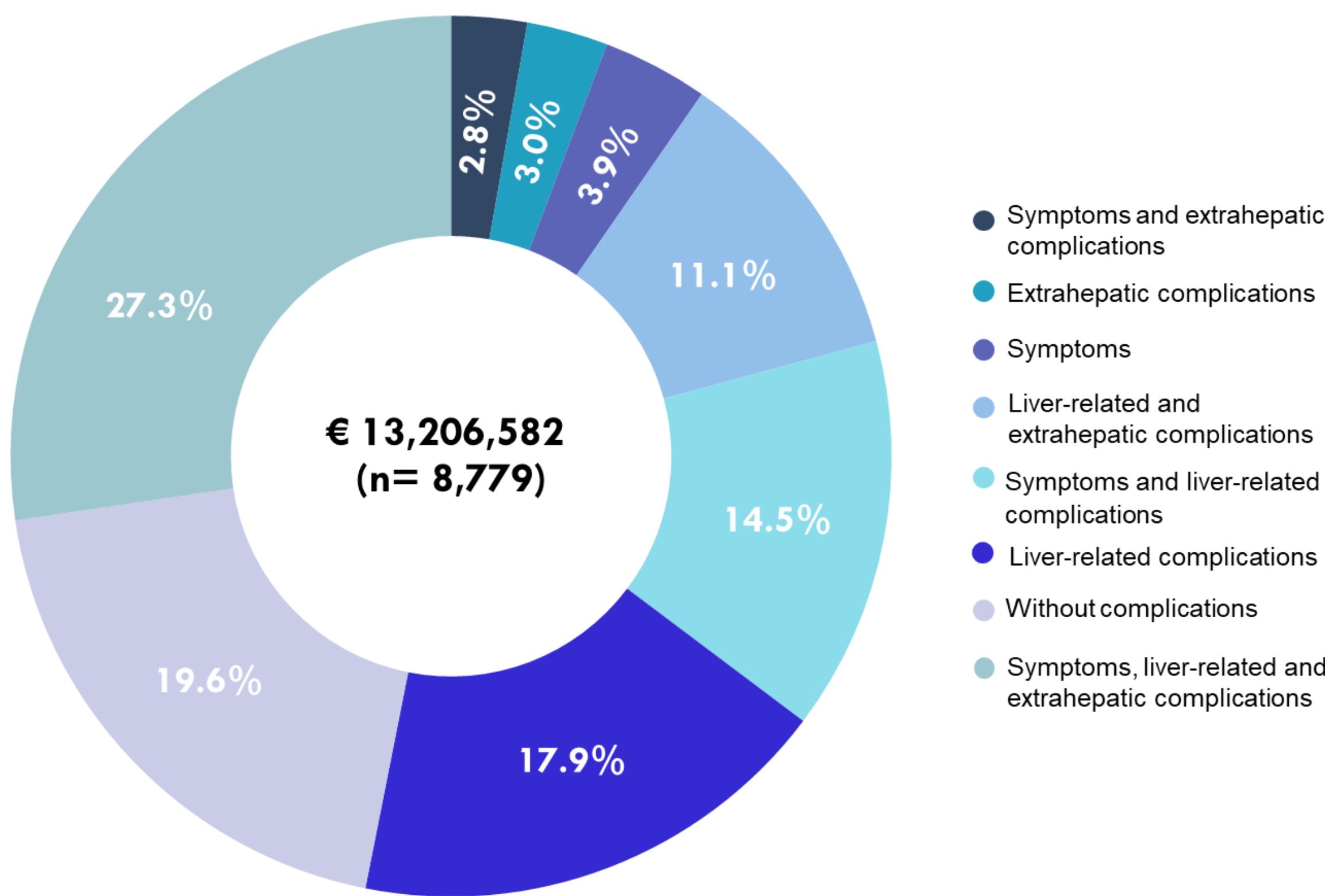
- A total of 8,779 patients with diagnosed PBC in Spain were estimated. Of these patients, 4,829 had no complications, 878 presented symptoms, 483 had associated extrahepatic complications, 307 had both symptoms and associated extrahepatic complications, 658 had liver-related complications, 483 had both symptoms and liver-related complications, 351 had both liver-related complications and associated extrahepatic complications, and 790 patients had symptoms, liver-related complications, and associated extrahepatic complications.
- The annual cost per patient increases with disease progression, ranging from €535 for patients without complications to €4,567 for those presenting with symptoms, liver-related and extrahepatic complications (Figure 2).

Figure 2. Annual cost per patient for each clinical situation



- Considering the overall population, the annual cost of managing PBC diagnosed patients was €13,206,582 (Figure 3).

Figure 3. Annual costs for the target population of managing PBC



- In the SA, the highest variation in total annual cost (€12,1M - €13,3M) was observed when key model parameters were adjusted. The most impactful factor was the cost associated with hepatocellular carcinoma and liver transplantation, followed by the proportion of patients presenting associated extrahepatic complications.

## CONCLUSIONS

- This analysis highlights the high economic impact of PBC in Spain, mainly driven by liver complications. Early diagnosis and effective treatment could significantly reduce the occurrence of these complications, thereby reducing the overall clinical and economic burden.

## REFERENCES

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