Rare Auto-immune Case Study

IRA Limits Treatment Options for Rare Autoimmune Diseases

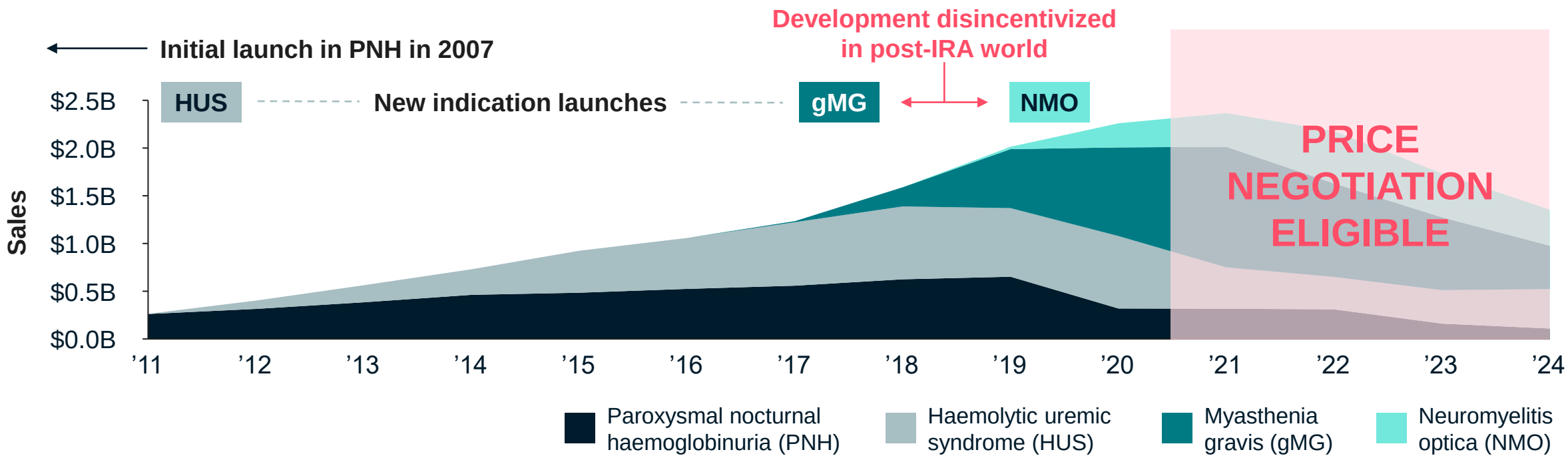
Autoimmune diseases happen when the body's immune system attacks and harms normal cells. There are more than 100 different autoimmune conditions, including rare ones. Treatment can help with symptoms and encourage remission from disease flares. Advances are often made by testing FDA-approved autoimmune drugs to see if they work for other autoimmune diseases. The IRA discourages this much needed research because it shortens the R&D time available for additional indications before drugs can be subject to Medicare price setting.

KEY POINTS:

- Many drugs for an autoimmune disease are tested and approved for use in different autoimmune diseases, including rare conditions.
- IRA's Medicare price setting exempts drugs only if they treat a single rare disease. That exemption is lost if a drug receives FDA approval for use for an additional non-rare or rare condition, including autoimmune diseases.
- The IRA also limits time and incentives to test medicines against other autoimmune diseases after a drug's initial FDA approval.

CASE STUDY: WORSE HEALTH OUTCOMES WITHOUT NEW TREATMENTS FOR PEOPLE WITH RARE AUTOIMMUNE DISEASES

If the IRA had been in effect when a certain drug was approved in 2007, the R&D leading to two additional FDA approvals for other rare diseases ten years later may never have occurred. Patients would have experienced significantly worse health outcomes and more hospitalizations and blindness.



Annual Impact of the IRA Disincentivizing Development of Novel Autoimmune Therapies

Rare progressive muscle weakness
(~48k patients in US)

- **1st FDA-approved treatment for patients in more than 60 years**
- ~900 patients per year may not achieve remission
- ~530 fewer hospital visits per year

Rare, progressive inflammatory vision loss
(~6k US patients)

- **~180 patients a year would become permanently blind or visually impaired**
- ~350 patients a year would experience a relapse
- 13x more likely to experience a relapse

If the IRA had been passed before the development of the novel autoimmune therapy, the manufacturer may NOT have conducted trials for two additional rare diseases, **significantly worsening treatment outcomes for ~1500 people with debilitating rare diseases a year**