IRA Threatens Advancement in Fight Against Rare Cancers

Thirty million people in the US have a rare disease, including many rare cancers. The vast majority do not have treatments available to help. Research and development for therapies to treat rare diseases is complex due to the small number of people affected, less medical knowledge about the underlying causes and natural history of the diseases, and variations within the subtypes of diseases, among other issues. Advances are often made by testing FDA-approved rare disease drugs to see if they work for other rare diseases. The IRA may discourage this much needed research by making rare disease approved drugs subject to Medicare pricing if the drug is approved to treat another rare disease or even if the drug receives FDA designation for just one additional rare disease.

Key Points

- Many cancer drugs are further researched, developed, and approved for use in different cancers, including rare cancers.
- IRA exempts drugs from price controls only if they treat one rare disease. That exemption is lost if a drug receives an additional FDA approval for a non-rare or rare indication or a designation for more than one rare disease, including cancers.
- According to NORD, 95% of rare diseases have no treatment – over 9,000 individual conditions
- From 1990 to 2022, 35% of orphan drugs had FDA approval for multiple conditions and one in six were approved only for rare conditions. Half of subsequent FDA approvals on orphan drugs came five years or after the initial FDA approval[1].
- IRA may discourage focus on rare cancers, as incentives are distorted to encourage initial approvals that impact the largest possible patient population.
- IRA's Medicare pricing also limit time and incentives to test medicines against other cancers after a drug's initial FDA approval.

People Receiving Cancer Medicines Approved for More than One Rare Cancer (2022):

For one medicine FDA approved to treat multiple rare cancers, more than a third of the people using it could lose access because the IRA penalizes new indications of FDA approved drugs for rare cancers and other diseases.

More than one in three people with rare cancers could lose the benefit of novel treatments due to the IRA, thereby facing higher burden of illness and premature death.

Abbreviations: IRA = Inflation Reduction Act

Source: [1]Miller, K.L., Lanthier M. (January 2024). Orphan Drug Label Expansions: Analysis Of Subsequent Rare And Common Indication Approvals. Health Affairs. Available at: https://www.healthaffairs.org/doi/epdf/10.1377/hlthaff.2023.00219.

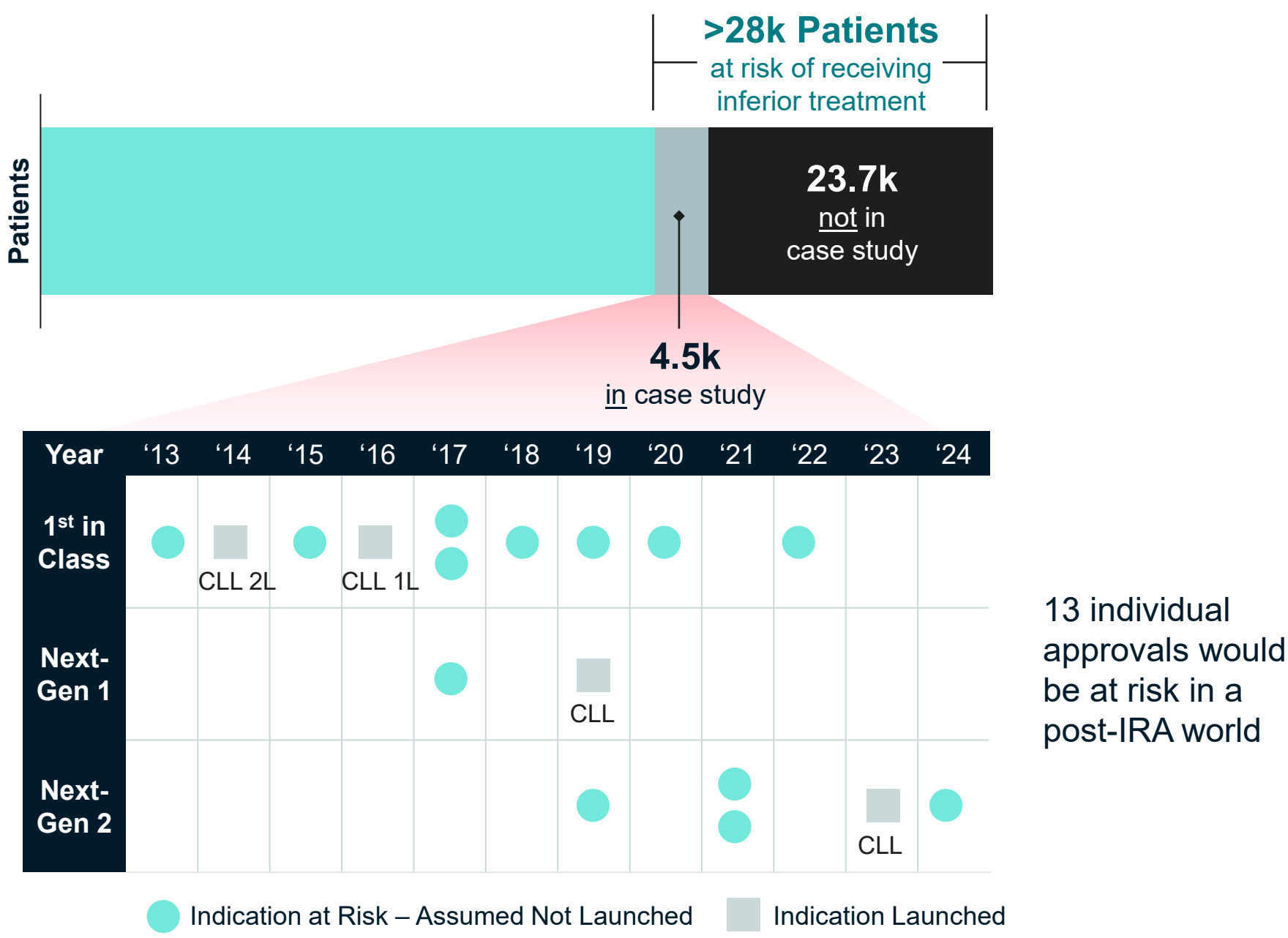
IRA Threatens Advancement in Fight Against Rare Cancers (cont'd)

CASE STUDY SCENARIO: 240,000 Life Years Lost for Patients with Rare Blood Cancers

BTKi inhibitors and chronic lymphocytic leukemia (CLL)

BTKi inhibitor treatments that treat CLL are also approved for use in other, rarer cancers. The IRA may discourage manufacturers in pursuing other indications, putting future research, FDA approvals, and access to these therapies at risk. The benefit to patients not realized in this example amounts to nearly a quarter million cancer-free life years over the course of a decade for patients with devastating conditions such as MCL.

People Receiving Cancer Medicines Approved for More than One Rare Cancer (2022):



13 individual approvals would be at risk in a post-IRA world

Anyone diagnosed with a rare cancer **risks losing treatment advances** from loss of cancer drugs approved for more than one rare cancer.

*30% derived from peak sales by indication

Abbreviations: IRA = Inflation Reduction Act; CLL= Chronic lymphocytic leukemia; BTK = Bruton tyrosine kinase; MCL = Mantle Cell Lymphoma