

# Before You Invest:

Lessons From What Patient Data Reveals About Drug Programs



by Pawel Zawadzki, PhD



## Case Study

---

# The WRN helicase

# \$400M+

Invested by multiple companies

## A Costly Failure

Despite massive investment from multiple companies, clinical trials for WRN inhibitors have repeatedly shown minimal benefits for cancer patients.

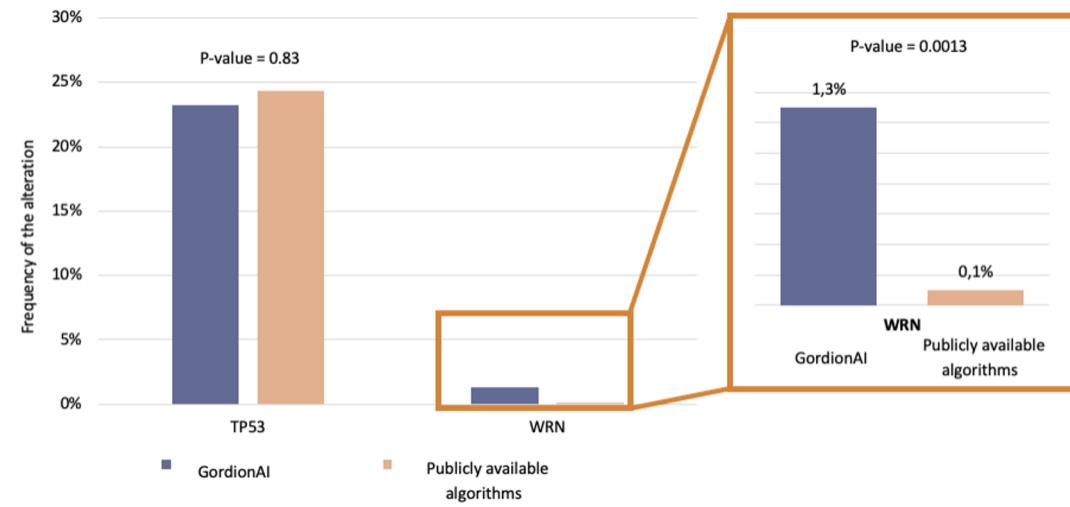
This outcome highlights a stark disconnect between strong data from preclinical models and the complex reality of patient responses.

# What the Patient Data Revealed

Deep, AI-augmented analysis of patient data uncovered a error produced by commonly used algorithm:

- A previously undetected type of WRN mutation was identified by Gordion technology.
- This mutation proved that WRN is not essential for cancer cell survival, hence a poor drug target. Actually, it appears WRN is rather a tumor suppressor!
- This single, patient data-derived finding directly explained the widespread failure of the clinical trials of WRN inhibitors.

Mutations inactivating both copies of a gene



For TP53, both the publicly available and the Gordion algorithm detected the same number of mutations. However, the commonly used algorithm was blind to mutations affecting WRN.

# The Take-Home Message:

## **Absence of evidence, is not evidence of absence.**



### The flawed assumption

Publicly available algorithms failed to detect the key mutation type. Investment teams mistakenly concluded WRN was an attractive target because the invalidating evidence was "absent."

### AI warning

This is a critical lesson for all AI users: the origins of AI-generated insights can be unclear. However, even a greater challenge is assessing what your models might be overlooking.

## Strategic Recommendations

---

# From Insight to Action

# Consult Patient Data for Reliable Outcomes:

- ✓ Be skeptical about computational tools, as each has its own limitations. A proper AI stack can eliminate these problems.
- ✓ Target selection is still oncology's biggest single-point failure. Patient data and well-designed AI are the only reliable way to know which targets truly matter.
- ✓ But do not delay AI adoption, as others will outcompete you. Select your AI partner wisely, as this will define your future.



**Medicines intended to work in  
patients should be developed  
with patient data in mind.**

Full WRN report available at:

<https://www.gordion.bio/portfolio-optimization>