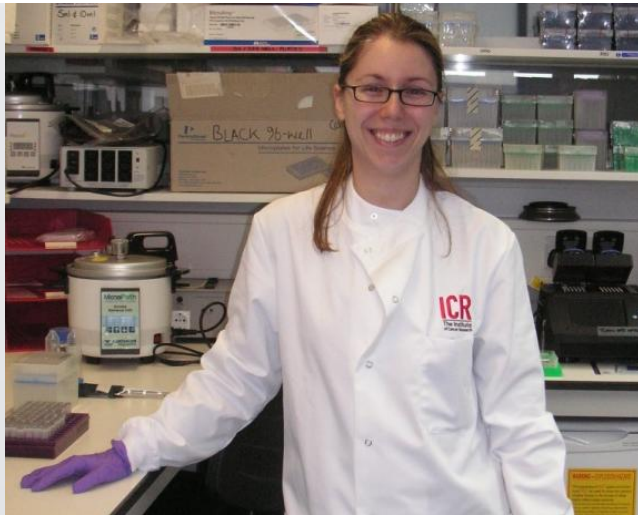


# RobsARTTT update 2012

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**ICR**  
The Institute  
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# Sarcoma Molecular Pathology Team



- Study Soft Tissue Sarcomas
- Speciality in Rhabdomyosarcomas (the biggest group)
- Also extended our research to other teenage sarcomas inc. Desmoplastic Small Round Cell Tumours (DSRCTs)

# What our research does

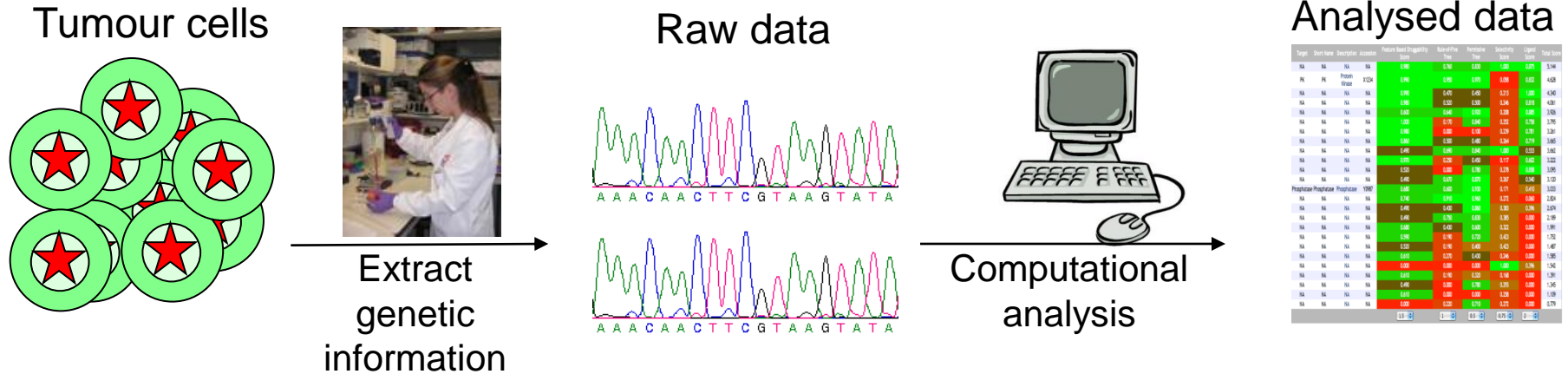
- Finds new therapies that work better & that have lower general toxicity

Recently, we identified a new potential target for treatment of rhabdomyosarcoma

- Results from our laboratory have direct relevance to patient treatment

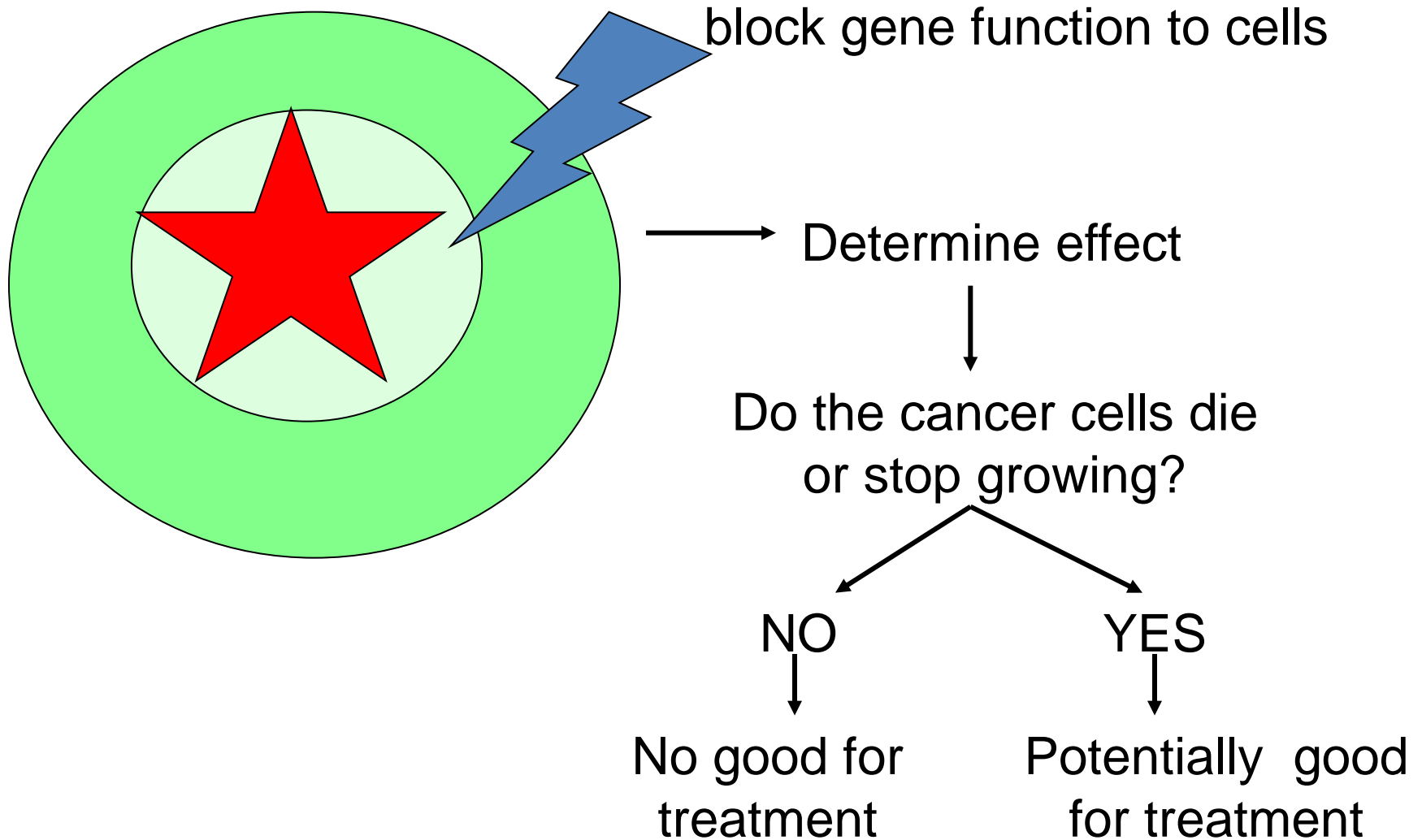
We have shown that a single genetic test can help classify patients into risk groups. Potential outcome: more appropriate treatment

# Understanding DSRCTs at the molecular level



**Conclusion:** We now know how a DSRCT tumour looks like at the molecular level for ALL of our genes (estimated to be ~20000-30000 genes).

# Understanding DSRCTs at the molecular level



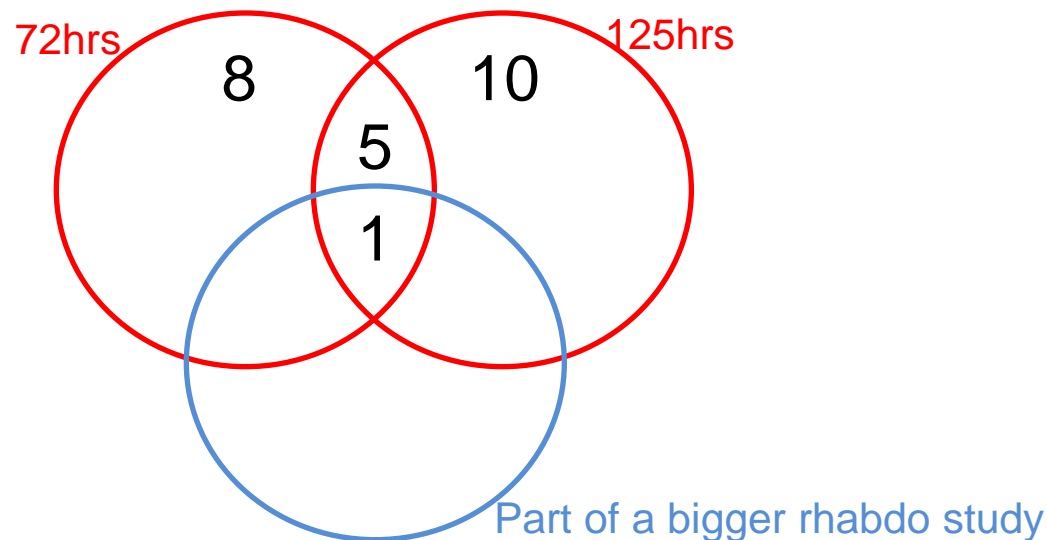
# Understanding DSRCTs at the molecular level:

- Experimental design:
  - We have found 77 different genes that could be important in DSRCTs
  - If we block the function of these 77 genes, do they stop DSRCTs from growing?

# Understanding DSRCTs at the molecular level:

## 📌 Results:

- We looked at 2 different time points after individually blocking the 77 genes - a number of which reduced tumour cell growth at 72hrs and/or 125hrs and therefore provide potential drug targets, and one is also being currently investigated for rhabdomyosarcoma.



## Conclusions:

- We have come up with a list of potential drug targets for DSRCTs.
- We can include the DSRCT cell line in the investigation of one of these targets as part of a rhabdomyosarcoma study.

# Summary

- Our work has potential clinical impact:
  - New target for rhabdomyosarcoma treatment
  - New genetic test for rhabdomyosarcoma classifies patient risk
- We have extended our work to DSRCTs (Thanks RobsARTTT):
  - Obtained preserved tumour and cell line for DSRCT research
  - Analysed all the genes in DSRCT – useful for current and future work
  - Modified activity of 77 different genes in DSRCT and assessed cell growth – identified short list of potential therapeutic targets
  - One genetic target is being investigated further and may also be a relevant drug target for rhabdomyosarcoma
  - This drug target will be investigated in conjunction with more standard therapies and pursued through our hospital and international links



# Thank you for your support!

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