Innovative Trial Approaches

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Problem adolescent T2D recruitment to PIPs

- Numbers of drugs in the pipeline/PIPs
- Limited population Europe/USA
- Poor diagnostic definitions
- Difficult population to engage in clinical research

Needs of diabetes academics caring for adolescents with T2D: availability of new products

- Limited treatment options - diet, exercise, metformin, insulin
- Rapid clinical development
- More aggressive disease with high rates of complications

Critical decision points which need to be addressed

- Patient on metformin with poor HbA1c control
- Will new oral agents, gliptins, SGLT2 inhibitors, avoid the need to introduce insulin therapy?
- If introduction of insulin therapy is inevitable or weaning off insulin is desirable are there agents such as GLP-1 agonists which could provide improved control or have other clinically advantageous outcomes such as reduced weight gain…?
- How could we derive an evidence base for triple therapy (e.g. Metformin, Gliptin, Insulin or GLP-1 agonist) which may be required to prevent profound morbidity in these subjects

How do we rapidly get to the point where we can apply drugs developed in adults to fulfil these aims in the Adolescent population?

- What is the bottom line for adolescents which can ensure safety efficacy for application in children which can be inferred from adult studies?
  - PK data – Age , sex, pubertal stage
  - Efficacy – postulate why such drugs would not be effective or safe in younger populations?
  - Identify outcomes which might with a limited exposure in adolescents provide appropriate safety/outcome variables
  - To what extent could European /USA academics network postmarketing surveillance convince regulators that safety and efficacy of provisional supported by PIP data are robust and preserve patient safety