Sanfilippo (MPS IIIA) disease is one of the most common MPS diseases, resulting from missing enzymes in the body’s cells. This leads to complex sugar accumulation in brain cells affecting their function. Since replacement enzyme cannot access the brain, there is no cure for patients. All the body’s cells come from simple cells that rely on signals from other cells or from themselves to grow. One of the signals important for growth and development into specialised cells are the complex sugars that accumulate in Sanfilippo disease. Using simple stem cells from skin and turning these into brain cells in the laboratory we hope to improve our knowledge of the specific steps involved in brain cell development. By further understanding how the cell environment affects growth and transition into brain cells in both cell culture and in Sanfilippo mice, will determine whether these stem cells have a future therapy option for Sanfilippo patients.