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Mycophenolate mofetil achieves remission in AAV

MMF is non-inferior to cyclophosphamide for achieving remission in people AAV, but results in higher relapse rate

INTRODUCTION

ANCA-associated vasculitis (shortened to AAV), is a rare group of diseases that are very serious, and can be life-threatening. These diseases are linked to a type of autoantibody called ANCA. An antibody is a protein that the normal healthy immune system makes to attack foreign substances in the body, such as viruses or bacteria. In people with AAV the body makes antibodies that attack its own tissues – these are called autoantibodies. In AAV, the ANCA autoantibodies affect the white blood cells and cause damage to the small blood vessels. Any part of the body can be affected, but AAV most often affects a person's kidneys, lungs, joints, nerves, and may cause bleeding in their nose and ears.

Cyclophosphamide is the normal treatment for people with AAV, but it has several possible side effects, including infertility, infection and cancer. Mycophenolate mofetil is another drug that is usually used in other autoimmune diseases, but it could also be useful in people with AAV.

WHAT DID THE AUTHORS HOPE TO FIND?

The authors wanted to find out whether mycophenolate mofetil could be used as the first treatment (induction treatment) instead of cyclophosphamide in people with AAV.

WHO WAS STUDIED?

The study included 140 people newly diagnosed with AAV from six countries in Europe, Australia and New Zealand. Most people were over the age of 18, but there were also eight children included. People were not allowed to take part if their disease was imminently life-threatening, if they had rapidly declining kidney function, or if they had already received more than 2 weeks of treatment with cyclophosphamide or mycophenolate mofetil.

HOW WAS THE STUDY CONDUCTED?

This was an open-label, randomised controlled trial, which means that patients were assigned by chance to one of two treatment groups. Using chance in this way means that the groups will be similar and will allow the variable or treatment under investigation to be compared objectively. 'Open-label' means that both the people in the trial and their doctors knew which medicine they were taking.

The first group received mycophenolate mofetil, and the second group received cyclophosphamide. Everyone also received a steroid treatment over the 6 months of the study. Once remission was achieved, people from both groups were switched to another drug called azathioprine.

There were clinic appointments at regular intervals to assess whether people's AAV had got better. People were also assessed at extra appointments if they suffered a relapse, or their disease worsened.

WHAT WERE THE MAIN FINDINGS OF THE STUDY?

The study found that both mycophenolate mofetil and cyclophosphamide could get people to remission. However, after remission and switching to azathioprine, more people who had taken mycophenolate mofetil suffered from relapses than those who had taken cyclophosphamide (33% compared to 19%). The numbers of people getting serious infections were similar between the two groups.

ARE THESE FINDINGS NEW?

Yes, this is the first proper study to show that remission rates with mycophenolate mofetil are non-inferior to cyclophosphamide, but that mycophenolate mofetil may be associated with a higher rate of relapse.

WHAT ARE THE LIMITATIONS OF THE STUDY?

There are some limitations to the study – firstly that it was open-label, which can mean that there is bias if patients or doctors expect a certain result from one of the medicines being tested. However, the authors are confident that the results do not show any bias in this trial. The trial had quite a short follow-up period (18 months), which means it is not possible to say how well the two drugs work long-term, or if there are delayed safety effects, and in other studies mycophenolate mofetil has been shown to be less good than a drug called azathioprine for maintaining remission. However, there is limited evidence for using azathioprine as an induction therapy in AAV.

WHAT DO THE AUTHORS PLAN ON DOING WITH THIS INFORMATION?

No more studies are planned. This information will be shared with healthcare professionals so that they can consider using mycophenolate mofetil instead of cyclophosphamide in some people with AAV.

WHAT DOES THIS MEAN FOR ME?

If you have AAV that is not immediately life-threatening – and as long as your kidney function is not badly affected – these results suggest that there could be new treatment options for you.

Speak to your doctor if you have any questions or concerns about your disease or its treatment.

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