Meeting the challenge of disabling disease : animal research brings hope / Research for Health Charities Group.

Research so far has led to better treatment of these and many other disease, helping victims to live a better life. Doctors and scientists must be allowed to continue their vital work in order to conquer these and many other diseases.

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MEETING THE CHALLENGE OF DISABLING DISEASE

Animal research brings hope

RESEARCH FOR HEALTH CHARITIES GROUP

After decades of extensive research, much of it involving animals, medicine has succeeded in virtually eliminating many disabling diseases in the developed world. Polio and rubella (German measles) are striking examples of diseases that once brought untold misery. They have now been effectively controlled following the introduction of vaccines whose development depended on animal research. But other disabling conditions such as muscular dystrophy, multiple sclerosis and Parkinson's disease remain. Such diseases can still have appaling effects on sufferers' quality of life, and on that of their families.

PEOPLE WITH DISABLING DISEASE SPEAK OUT

Andrew Blake suffers from Friedreich's ataxia, an incurable and ultimately fatal inherited disease in which the nervous system fails to regulate the posture of the body and the strength and direction of limb movements. It first reveals itself in apparently healthy children by causing unsteadiness and lack of co-ordination. This is followed by speech difficulties, tremor of the hands and head and, finally, curvature of the spine. The brain itself remains healthy, but is trapped inside a crippled body. At the age of 28, Andrew has been confined to a wheelchair for ten years.

Andrew explains why he feels so strongly that research should be allowed to continue.

"Not a day passes without me dreaming of living a normal life. The only hope for people like me who suffer from serious illness is a major breakthrough in medical research. For some of us, this is the only thing that makes life worth living.

"Even if we do not benefit, and it is getting late for that, those who are now children and future generations may be spared the pain and misery I have endured."

LEARNING FROM WHAT HAS ALREADY BEEN ACHIEVED

Reducing the anguishing disablement that is suffered by Andrew and many others like him is a major priority of medical research. Sufferers are coping bravely with their difficulties. But we should not expect them to continue with this burden. Much has already been achieved in improving quality of life in these difficult conditions. Far more needs to be done to find a cure and prevention.

Millions of pounds are now being spent on research into such conditions as multiple sclerosis, muscular dystrophy, Parkinson's disease and arthritis. It is vital that this work be allowed to continue.

Parkinson's disease provides a clear example of how animal research underpins progress against disability. It was experiments involving rats that first led scientists to discover that the disease is caused by lack of an essential chemical substance, dopamine, deep within the brain. Animals were then also used to test drugs that replace this substance. The result is that Parkinson's disease sufferers can now be helped by doctors. The condition is not curable, but its distressing symptoms can be alleviated and life prolonged.

Scientists and doctors are also working on the idea that one form of Parkinson's disease can be reversed by transplanting small amounts of brain tissue. The treatment works in rats and is now being assessed

in small groups of patients. Both of these developments bring renewed hope to the many thousands of people (some of them in early middle-age) whose lives are blighted by this cruel disease.

Multiple sclerosis (MS) is a common disease, affecting about 80,000 people in the UK, which results in impaired sensation and difficulties in movement. The nerves are normally surrounded by a sheath which acts like the insulation around an electrical cable. In MS, the insulating sheath becomes scarred, with the result that messages from the central nervous system to different parts of the body are interrupted. It is diagnosed between the ages of 20 and 40 and affects more women than men. It is not a fatal disease. Typically, MS sufferers experience debilitating attacks of the disease, followed by periods of remission. But the attacks become more frequent and severe, and about half of all people with MS eventually have great difficulty in walking.

Information from animal experiments has helped establish that MS in people is essentially an "autoimmune" disease in which the defences of the body for some reason turn on themselves. Specific cells that attack myelin have been identified in rodent models of MS and, subsequently, they have now also been found in human blood samples. This is a good example of how animal research advances our understanding of disease.

The precise cause of MS has still to be discovered, and no generally effective therapy is available. But scientists are following several promising leads. They are investigating the potentially crucial role of cells that produce myelin and so may be able to repair the insulating nerve sheaths damaged by the disease. The important work is being done in rats.

Scientists have also now succeeded in inducing a condition similar to MS in rats and mice and have treated it successfully using antibodies that "switch off" the disease process. Following the animal research, this potential treatment is now to be tried in the USA and the UK in small studies involving patients.

Muscular dystrophy is a group of related conditions that lead to wasting of the muscles. Around 20,000 people in the UK are affected. The most common and devastating form of the disease is Duchenne muscular dystrophy. Scientists have now found the defective gene that is the cause of Duchenne, and have identified the protein, called dysrophin, that is missing in those who suffer from the disease. Following the discovery, research is now being targeted specifically at finding a treatment.

Animals are essential to this process and the mouse, in particular, has a vital role to play. Scientists have shown that the exact genetic abnormality found in males with Duchenne is also found in some mice. The crucial difference is that mice with the gene defect do not develop wasted muscles, even though they lack dystrophin.

If we can find out how mice preserve muscle function without dystrophin, that should give us clues towards how humans might also be treated. However, there is another major possibility which is to insert healthy muscle cells, grown in tissue from a donor, into the wasted muscles. Much work is already being undertaken on this possibility, using the affected mice to determine both the positive and negative aspects of such a potential treatment before undertaking large scale trials on humans.

Arthritis is a range of diseases that cause inflammation of the joints, leading to pain, swelling and disability. Many drugs can be used to control these symptoms. They include aspirin, steroids and other anti-inflammatory drugs, all of which have been extensively tested on animals. But doctors are not yet able to reverse the destructive processes that underlie the disease.

Ankylosing spondylitis is a severe and crippling form of arthritis, affecting the spine in particular. Two people in every thousand suffer from it. Recently, American researchers have discovered that the transfer of a human gene into rats can imitate the symptoms of the disease in humans. This progress should aid the development of more effective therapies.

The use of such animal models is also crucial to understanding why joints are gradually destroyed in other forms of the disease. In rheumatoid arthritis, for example, animal experiments are beginning to show what happens when the immune system mistakenly attacks our own bodies.

Animal research is also vital in improving the treatment of osteoarthritis, a common but distressing condition which affects the majority of the elderly population. Artificial hips, which are used to replace joints destroyed by osteoarthritis, are often considered among the most valuable contributions that modern medicine has made to the relief of pain and disability.

THE PROMISE OF GENE THERAPY

Many disabling diseases, such as cystic fibrosis, Duchenne muscular dystrophy and Friedreich's ataxia, are caused by absent or defective genes. As we learn which genes are involved, it has become possible to think of curing these conditions by transferring healthy genes into someone with the defect.

It would not be ethical to use such untried techniques on patients, since the risks and benefits are still unknown. But scientists have now begun to develop gene transfer techniques in rodents. The difficulties involved in this pioneering technique should not be underestimated. But the use of laboratory animals means that the results of each slow step forward can be carefully assessed. And such research now offers a glimmer of hope to those who suffer from many disabling diseases.

Much of the vital medical research undertaken does not involve the use of animals. Where it does, it's because they are absolutely essential to help doctors learn how to prevent or relieve real human suffering.

Here are some examples of research in which animals have played an important part:

★ To improve the success rate of artificial bone implants, specialists are working out how bone cells interact with artificial bone and become attached to it. Their research will help bone-substitute materials to be designed.

★ Cystic fibrosis is caused by a defective gene and affects mainly the lungs and also the pancreas leading to progressive decline in their function. At the moment heart-lung transplantation is regarded as the only chance of survival for some children and young adults crippled by this disease, until gene therapy becomes a realistic alternative.

★ Improving treatment of injuries to the nervous system is one of the most complex problems facing doctors and surgeons. Accidental severing of nerves is quite common in road and industrial accidents and only a minority of patients achieve a good recovery because too few of the fibres grow back again.
